

RANK TESTS FOR RANDOMLY RIGHT CENSORED BIVARIATE
LIFE-TIME DATA

By

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A DISSERTATION PRESENTED TO THE GRADUATE SCHOOL
OF THE UNIVERSITY OF FLORIDA IN PARTIAL FULFILLMENT
OF THE REQUIREMENTS FOR THE DEGREE OF
DOCTOR OF PHILOSOPHY

UNIVERSITY OF FLORIDA

1992

To my parents and teachers

ACKNOWLEDGEMENTS

I would like to thank Professor P.V. Rao for his guidance during all the years of my research to complete the degree of Doctor of Philosophy. Without his enormous patience and encouragement this would not have been possible. I consider myself extremely fortunate to have had the opportunity to work with him. I would like to thank Professors Randles, Littell, Ghosh, and M. Rao for their encouragement and advice while serving on my committee.

Whenever I think of the time spent at the Department of Statistics here, I am reminded of Professor Scheaffer who made it possible for me to come here as a graduate student in spite of all the obstacles. In my six years as a student here, I learned from all my professors. I would also like to specially thank Dr. Littell for all the support while I worked as a consultant for three years in IFAS. I would also like to express my gratitude to my colleague in IFAS, Steve Linda for his continuous help during the preparation of this document and also for teaching me about computers and statistical computing.

Also, thanks go to my parents for all their encouragement and to my beloved husband Sudeep, who was always there to support me. There are numerous other friends who made my years in graduate school so memorable and wonderful. Their friendship will never be forgotten.

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Abstract of Dissertation Presented to the Graduate School
of the University of Florida in Partial Fulfillment of the
Requirements for the Degree of Doctor of Philosophy

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August 1992

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The problem of testing for difference in survival times using randomly right censored bivariate data has been considered by several researchers. Among them are Woolson and Lachenbruch who proposed a class of statistics for testing the null hypothesis (H_0) of bivariate symmetry in Biometrika, 1980. Popovich and Rao proposed an alternative to the Woolson-Lachenbruch class of statistics in Communications in Statistics 14(9), 1985. The statistics in this class provided a means of performing conditionally distribution-free exact tests of H_0 . Dabrowska used a counting process representation to derive the asymptotic distributions of members of Woolson-Lachenbruch class of statistics under fixed and contiguous alternatives in the Journal of the American Statistical Association 85, 1990.

In this dissertation, counting process representations are used to study the properties of Popovich-Rao class of statistics. Efficacies of the members of this class are derived using a contiguous sequence of parametric alternatives. The form of the

statistic with maximum efficacy, to be called optimal statistic, is derived for both Popovich-Rao and Woolson-Lachenbruch classes.

Efficacies of the optimal statistics are compared with the other members using a log-linear model. Asymptotic Relative Efficiency (ARE) of the optimal Popovich-Rao statistic with respect to optimal statistics in other classes improves as the correlation between the survival times increases. Since the form of optimal statistics involve weights which depend on the unknown joint distribution of survival times in a complicated manner, a method is proposed for estimating the weights from observed data.

The performance of optimal statistics is evaluated in terms of powers using a simulation study with several survival distributions, censoring patterns and sample sizes. Simulation studies indicate that the power of the optimal statistic in each class is higher than that for other members in the same class. The results of power comparisons are consistent with those obtained from ARE comparisons. Furthermore, the powers indicate that the optimal weights are robust with respect to the specifications of underlying parameters.

CHAPTER 1

INTRODUCTION

1.1 Censored Life-Time Data

In many experiments, one is interested in comparing two drugs to see if there is a difference in the lengths of time the drugs (treatments) are effective, or if there is a difference in the lengths of time the drugs are taking to cure a certain disease. The time period during which a treatment is effective is called the survival time or the lifetime of the treatment. If, for a particular subject, the treatment is still effective at the time of termination of the experiment, then the corresponding survival time, i.e. the time period in which a treatment is effective, is longer than the observation time for that subject. In that case the true survival time is unknown and the survival time is said to be right censored at the length of time the subject was under observation. Sometimes at the time of the first observation of a treated subject, the treatment is already ineffective so that the corresponding survival time is not observable, but known to be less than the observation time of that subject. In that case the survival time is said to be left censored at the observation time.

It should be noted that censored data can arise in cases where responses are not measured on a time-scale. Miller (1981) gives an example from Leavitt and Olshen (1974) where the measured response is the amount of money paid on an insurance claim on a certain date. For this example, an uncensored response would be the

amount paid on the total claim, while a right censored response would be the amount paid to date if the total claim is unknown.

Censoring can arise in different situations due to experimental restrictions on the observation time. It can be broadly classified into three categories. Type I censoring occurs when the period of observation for each subject is preset to be a certain length of time T , called fixed censoring time. In such a case survival times longer than T would be right censored while those that are less than T would be observed and are failure times. Type II censoring occurs when the period of observation will terminate as soon as the r th failure is observed where r is a predetermined integer less than n , the number of subjects involved in the study. Then the $n - r$ survival times which are unobserved are right censored at the r th failure time. The third form of censoring is called random censoring. Random censoring occurs when subjects have their own observation times which need not be the same for all subjects. In most clinical trials, subjects enter the study at different times and each subject's observation time is the elapsed time between entry and the end of the study. Clearly, if a subject's survival time is greater than the subject's observation time, then a right censored survival time results; otherwise, a failure time is observed for the subject.

1.2 Overview of the Manuscript

This dissertation is concerned with the problem of testing the equality of two survival distributions using matched pairs of randomly right censored survival times. Survival times may occur in pairs either naturally or by experimental design (Kalbfleisch & Prentice, 1980). Examples of natural matched pairs are twin studies or

the studies in which two survival times are recorded on the same individual or piece of equipment. The other case of paired data arises in a matched pair study where individuals sharing certain characteristics are assigned to a pair. For comparing the effect of two drugs, patients with similar characteristics are paired, then each receives a drug at random and are kept under observation.

In what follows, it is assumed that members from a given pair have equal censoring times. When survival times are recorded on the same individual, censoring time is the same for both observations in the pair. But, when pairs are matched through experimental designs, censoring time can be different for the two members in the pair. In that case, the minimum of the two censoring times is taken as the common censoring time for the pair. It is also assumed that censoring is independent of the treatments under study and the censoring, if it occurs, is right censoring.

Chapter 2 presents some key notation along with a brief literature review and a description of the general problem. Chapter 3 is devoted to developing efficacies of statistics belonging to specific classes considered by Woolson & Lachenbruch (1980) and Popovich & Rao (1985). The notion of an optimal statistic for each class is defined. A model is proposed in Chapter 4 for practical implementation of tests based on optimal statistics. The results of a simulation study and an illustration with real data are presented in Chapter 5.

The referencing system in this manuscript will follow the convention of numbering equations, theorems and lemmas within section. However, Tables and Figures will be numbered within chapter. Theorems and lemmas are counted within a section and are numbered independently of one another. For example, the first theorem in the second section of Chapter 2 is Theorem 2.2.1, and the first lemma is Lemma 2.2.1.

CHAPTER 2

FORMULATION OF THE PROBLEM

2.1 Introduction

Some notation needed to develop the topic of this dissertation is described in Section 2.2 while Section 2.3 describes the general problem with a brief historical survey of the relevant literature. Section 2.4 contains a description of the specific objective of this dissertation.

2.2 Notation

Let (X_1, X_2) be a bivariate random vector with a continuous density function $\psi(x_1, x_2)$. Let C be an independent continuous random variable with cumulative distribution function (c.d.f.) G and survival function $\bar{G} = 1 - G$. We shall refer to X_1 and X_2 as the survival times and to C as the censoring time. The observed survival times are

$$Y_1 = \min(X_1, C)$$

and

$$Y_2 = \min(X_2, C).$$

In addition to Y_1 and Y_2 , we also observe,

$$\delta_1 = \begin{cases} 1, & \text{if } X_1 \leq C \\ 0, & \text{otherwise.} \end{cases}$$

and

$$\delta_2 = \begin{cases} 1, & \text{if } X_2 \leq C \\ 0, & \text{otherwise.} \end{cases}$$

The indicator random variables δ_1 and δ_2 specify whether an observed survival time is a censored or a true survival time.

Let $Z = Y_2 - Y_1$ be the observed difference between the survival times in a pair and $\epsilon = \text{sign}(Z)$, where

$$\text{sign}(Z) = \begin{cases} 1, & \text{if } Z > 0 \\ 0, & \text{if } Z = 0 \\ -1, & \text{if } Z < 0. \end{cases}$$

The censoring mechanism is such that $\epsilon = 0$ and $Z = 0$ whenever $\delta_1 = \delta_2 = 0$. Also, $\epsilon = 1$ whenever $\delta_1 = 1$ and $\delta_2 = 0$, and $\epsilon = -1$ whenever $\delta_1 = 0$ and $\delta_2 = 1$.

Let (X_{1i}, X_{2i}) and $C_i, i = 1, 2, 3, \dots, n$ be independently and identically distributed (i.i.d.) as (X_1, X_2) and C respectively. Also, let

$$Y_{ki} = \min(X_{ki}, C_i),$$

$$Z_i = Y_{1i} - Y_{2i} \quad \text{and} \quad \epsilon_i = \text{sign}(Z_i),$$

for $k = 1, 2, \quad i = 1, \dots, n$.

An observed (Y_{1i}, Y_{2i}) can be classified into one of five classes based on the ϵ_i (the sign of Z_i) and $(\delta_{1i}, \delta_{2i})$ (the censoring pattern of the (Y_{1i}, Y_{2i})). The classes are defined by the sets of indices :

$$B_1 = \{i : \epsilon_i = 1, \delta_{1i} = \delta_{2i} = 1\},$$

$$\begin{aligned}
B_2 &= \{i : \epsilon_i = -1, \delta_{1i} = \delta_{2i} = 1\}, \\
B_3 &= \{i : \delta_{1i} = 1 \text{ and } \delta_{2i} = 0\}, \\
B_4 &= \{i : \delta_{1i} = 0 \text{ and } \delta_{2i} = 1\}, \\
B_5 &= \{i : \delta_{1i} = \delta_{2i} = 0\}.
\end{aligned} \tag{2.2.1}$$

If Y_{1i} and Y_{2i} are both uncensored, then for $i \in B_1 \cup B_2$, $Z_i = X_{1i} - X_{2i}$ is the difference between the actual survival times for the i th pair. In that case, Z_i will be called an uncensored difference and the corresponding pair of observed survival times will be called an uncensored pair.

If exactly one of the survival times in (Y_{1i}, Y_{2i}) is censored, then $i \in B_3 \cup B_4$. The corresponding Z_i will be referred to as a censored difference and the pair will be called a singly censored pair. In particular, it should be noted that if $i \in B_3$, then $Z_i < X_{2i} - X_{1i}$ i.e. the observed difference is less than the true difference. Similarly, the observed difference is greater than the true difference for $i \in B_4$. Finally, if $i \in B_5$, then $Z_i = 0$ and we shall refer to (Y_{1i}, Y_{2i}) as a doubly censored pair.

Let

$$\begin{aligned}
N_u &= \text{Total number of uncensored pairs} \\
&= \sum_{i=1}^n \delta_{1i} \delta_{2i} \left(\frac{1 + \epsilon_i}{2} \right) + \sum_{i=1}^n \delta_{1i} \delta_{2i} \left(\frac{1 - \epsilon_i}{2} \right) \\
&= N_{u+} + N_{u-},
\end{aligned}$$

where N_{u+} and N_{u-} are the total numbers of uncensored positive and negative differences, respectively,

$$\begin{aligned}
N_c &= \text{Total number of singly censored pairs} \\
&= \sum_{i=1}^n \delta_{1i} (1 - \delta_{2i}) + \sum_{i=1}^n (1 - \delta_{1i}) \delta_{2i} \\
&= N_{c+} + N_{c-},
\end{aligned}$$

where N_{c+} and N_{c-} are the total numbers of singly censored positive and negative differences respectively, and

$$\begin{aligned} N_d &= \text{Total number of doubly censored pairs} \\ &= \sum_{i=1}^n (1 - \delta_{1i}) (1 - \delta_{2i}). \end{aligned}$$

The various statistics to be considered in this manuscript will be represented using the following four counting processes :

$$N_j(t) = \sum_{i=1}^n N_{ji}(t) \quad j = 1, 2, 3, 4,$$

where

$$N_{ji}(t) = I[|Z_i| \leq t, i \in B_j], \quad i = 1, 2, \dots, n, \quad j = 1, 2, 3, 4,$$

are the processes that count the occurrences of uncensored and singly censored absolute differences.

Following Dabrowska (1990), we define the subsurvival function F_j as

$$F_j(t) = P(|Z_i| \geq t, i \in B_j), \quad j = 1, 2, 3, 4,$$

and

$$F(t) = \sum_{j=1}^4 F_j(t), \quad (2.2.2)$$

where $P(A)$ stands for the probability of the occurrence of event A . Note that $F_j(0) = P(i \in B_j)$ and

$$\sum_{j=1}^4 F_j(0) = 1 - p_0 \leq 1,$$

where p_0 is the probability of a doubly censored observation given by

$$\begin{aligned} p_0 &= P(i \in B_5) \\ &= \int \left\{ \int_u^\infty \int_u^\infty \psi(x, y) dx dy \right\} dG(u). \end{aligned} \quad (2.2.3)$$

The improper densities corresponding to the four subsurvival functions can be expressed as

$$\begin{aligned}
 f_1(t) &= -\frac{d}{dt}F_1(t) \\
 &= \int \overline{G}(u) \psi(u-t, u) du, \\
 f_2(t) &= -\frac{d}{dt}F_2(t) \\
 &= \int \overline{G}(u) \psi(u, u-t) du, \\
 f_3(t) &= -\frac{d}{dt}F_3(t) \\
 &= \int \left\{ \int_u^\infty \psi(u-t, y) dy \right\} dG(u),
 \end{aligned}$$

and

$$\begin{aligned}
 f_4(t) &= -\frac{d}{dt}F_4(t) \\
 &= \int \left\{ \int_u^\infty \psi(y, u-t) dy \right\} dG(u).
 \end{aligned} \tag{2.2.4}$$

Let $F_u(\cdot)$ denote the conditional survival distribution of $|Z_i|$ given the difference is uncensored i.e., given both Y_{1i} and Y_{2i} are uncensored. If p_u , the probability of observing an uncensored pair is nonzero then

$$\begin{aligned}
 F_u(t) &= P(|Z_i| \geq t, \mid i \in B_1 \cup B_2) \\
 &= \frac{(F_1(t) + F_2(t))}{P(i \in B_1 \cup B_2)} \\
 &= \frac{(F_1(t) + F_2(t))}{(F_1(0) + F_2(0))} \\
 &= \frac{F_u^*(t)}{p_u},
 \end{aligned} \tag{2.2.5}$$

where $F_u^*(\cdot)$ is the subsurvival function of $|Z_i|$ for $i \in B_1 \cup B_2$.

An unbiased estimate of $F_u(t)$ is given by the conditional empirical survival distribution,

$$\hat{F}_u(t) = \frac{\hat{F}_u^*(t)}{\hat{p}_u},$$

where $\hat{F}_u^*(\cdot)$ and \hat{p}_u are the sample analogs of $F_u^*(\cdot)$ and p_u respectively.

The notation described in this section will be used frequently in the remainder of this dissertation. Other notation necessary in the manuscript will be introduced as needed.

2.3 Literature Review

The problem of testing for the difference in survival times using randomly right censored bivariate data has been considered by several researchers. Among them are Woolson and Lachenbruch (1980) who proposed a class of statistics (to be called the WL-class) for testing the null hypothesis,

$$H_0 : \psi(x_1, x_2) \text{ is symmetric about } 0,$$

against the alternative,

$$H_1 : \psi(x_1, x_2) \text{ is not symmetric about } 0.$$

Let $t_{(1)} < t_{(2)} \dots < t_{(N_u)}$ denote the N_u ordered, absolute, uncensored differences, (i.e., the ordered $|Z_i|$ in $B_1 \cup B_2$) and $\epsilon_{(i)}$ be the ϵ_i corresponding to $t_{(i)}$. Furthermore, let $t_{(ij)}$, and $\epsilon_{(ij)}$ denote, respectively, the ordered values of the absolute differences in $B_3 \cup B_4$ which are contained in the interval $[t_{(i)}, t_{(i+1)})$ and the corresponding $\text{sign}(t_{(ij)})$, $j = 1, \dots, n_i$, $t_{(0)} = 0$, $t_{(N_u+1)} = +\infty$.

In the notation of Woolson and Lachenbruch (1980), a statistic in the WL-class can be expressed as

$$T = \sum_{i=1}^{N_u} \epsilon_{(i)} s_i + \sum_{i=0}^{N_u} \sum_{j=1}^{n_i} \epsilon_{(ij)} S_i \quad (2.3.1)$$

where s_i and S_i , $i = 1, 2, \dots, N_u$, are scores that depend only upon the form of $\psi(x_1, x_2)$, the joint density of (X_1, X_2) .

For small sample sizes, Woolson and Lachenbruch (1980) proposed a conditional test of H_0 using the observed scores s_i and S_i , $i = 1, \dots, N_u$. The conditional test is to be based on the permutation distribution of T generated by the 2^n equally likely assignments of signs to the scores. For large sample sizes, a test of H_0 can be based on the asymptotic normality of T , a property established by Dabrowska (1990).

Woolson and Lachenbruch (1980) developed the form of s_i and S_i under the assumption that (X_1, X_2) satisfies the log-linear model :

$$\begin{aligned} \log X_1 &= \theta + \eta_1 + \nu, \\ \log X_2 &= \eta_2 + \nu, \end{aligned} \quad (2.3.2)$$

where θ is an unknown parameter, η_1 , η_2 and ν are mutually independently distributed random variables and $D^* = \log X_1 - \log X_2$ has a specified density function ϕ that is symmetric about 0.

Censored data versions of Sign and Wilcoxon signed rank statistics correspond to using double exponential and logistic density for the density of D^* , respectively. These are the two cases explicitly considered by Woolson and Lachenbruch. In what follows, we will refer to the censored data versions of the Wilcoxon signed rank statistic and the Sign statistic as the WL Wilcoxon signed rank statistic and the WL Sign statistic respectively.

Although the WL-class of statistics provide a reasonable method for testing H_0 , due to the complicated functional form of the members of this class, it is difficult to

investigate their theoretical properties. Also, for small sample test, determination of the critical values needed for the permutation test can be tedious.

Popovich and Rao (1985) proposed an alternative to the WL-class of statistics. The statistics in this class (to be called the PR-class) provided a means of performing conditionally distribution-free tests of H_0 vs H_1 . Just as the statistics in the WL-class, the statistics in the PR-class are based on ranks of absolute differences $|Z_i|$, the signs ϵ_i and the censoring indicators $(\delta_{i1}, \delta_{2i})$.

The statistics in the PR-class can be expressed in the form :

$$T_n(N_u, N_c) = (1 - L_n)^{\frac{1}{2}} T_u^* + L_n^{\frac{1}{2}} T_c^*$$

where T_u^* and T_c^* are standardized (under H_0) versions of statistics T_u and T_c such that, T_u is a function of $(|Z_i|, \epsilon_i)$ for $i \in B_1 \cup B_2$ and T_c is a function of ϵ_i for $i \in B_3 \cup B_4$. The coefficient L_n is a function of N_u , and N_c such that,

$$(i) \ 0 \leq L_n \leq 1,$$

$$(ii) \ L_n = L + o_p(1) \text{ as } n \rightarrow \infty \text{ for some constant } L.$$

Any statistic for testing the symmetry of the conditional distribution of Z_i , given $i \in B_1 \cup B_2$, is reasonable for testing H_0 . One such statistic is the Wilcoxon signed rank statistic (Randles & Wolfe, 1979) applied to the Z_i , given $i \in B_1 \cup B_2$. The corresponding statistic has the form,

$$T_u = \sum_{i \in B_1 \cup B_2} \Psi(Z_i) R_i^+ \quad (2.3.3)$$

where R_i^+ denotes the rank of $|Z_i|$, in the set $\{|Z_i|, i \in B_1 \cup B_2\}$, and $\Psi(Z_i) = 1$ or 0 depending on $Z_i > 0$ or $Z_i \leq 0$, respectively.

Popovich and Rao (1985) selected Wilcoxon signed rank statistic as T_u due to its high efficiency for a wide variety of distributions of the Z_i and availability of tables of critical values for a large selection of sample sizes.

In their search for a candidate T_c , they showed that, conditional on $N_u = n_u$, and $N_c = n_c$, any statistic based on N_{c+} and N_{c-} is independent of any statistic based on $(|Z_i|, \epsilon_i)$ for $i \in B_1 \cup B_2$. In particular, the Sign statistic (Randles & Wolfe, 1979) based on

$$T_c = N_{c+} - N_{c-} \quad (2.3.4)$$

is conditionally independent of the statistic based on T_u in (2.3.3). Since T_c can also be used for testing H_0 , the linear combination

$$T_n(N_u, N_c) = (1 - L_n)^{\frac{1}{2}} T_u^* + L_n^{\frac{1}{2}} T_c^*, \quad (2.3.5)$$

is a member of the PR-class. The statistics in this class are conditionally distribution-free under H_0 . Since T_u and T_c are conditionally independent, exact critical values for $T_n(N_u, N_c)$ can be easily constructed from the critical values for Wilcoxon signed rank and Sign statistic which are widely available.

The exact forms of T_u^* and T_c^* at (2.3.5) can be determined by noting that under H_0 , the conditional means and variances of T_u and T_c are

$$\begin{aligned} \mu_u &= E[T_u \mid N_u = n_u, N_c = n_c] \\ &= \frac{1}{4} n_u (n_u + 1), \\ \sigma_u^2 &= \text{Var}[T_u \mid N_u = n_u, N_c = n_c] \\ &= \frac{1}{24} n_u (n_u + 1) (2n_u + 1), \end{aligned}$$

and

$$\begin{aligned} \mu_c &= E[T_c \mid N_u = n_u, N_c = n_c] \\ &= 0, \\ \sigma_c^2 &= \text{Var}[T_c \mid N_u = n_u, N_c = n_c] \\ &= n_c. \end{aligned} \quad (2.3.6)$$

Therefore,

$$T_u^* = [N_u(N_u + 1)(2N_u + 1)/24]^{-\frac{1}{2}} [T_u - N_u(N_u + 1)/4],$$

and

$$T_c^* = N_c^{-\frac{1}{2}} T_c.$$

Assuming some regularity conditions, Popovich and Rao (1983) also showed that the asymptotic null distribution of $T_n(N_u, N_c)$ at (2.3.5) is a standard normal distribution.

A simulation study was done by Popovich and Rao (1983) to compare the powers of tests based on four statistics in the PR-class to the power of WL Wilcoxon signed rank statistic. The statistics in the PR-class were determined by the following four forms of L_n .

When nothing is known about the underlying distribution, a reasonable choice for L_n is 0.5, leading to the first statistic

$$T - EQ = [(0.5)^{0.5} T_u^* + (0.5)^{0.5} T_c^*]. \quad (2.3.7)$$

Other choices of L_n considered by Popovich and Rao are, L_n proportional to N_c , resulting in the statistic

$$T - SQR = (N_u + N_c)^{-0.5} [(N_u)^{0.5} T_u^* + (N_c)^{0.5} T_c^*], \quad (2.3.8)$$

L_n proportional to N_c^2 , yielding the third statistic

$$T - SS = (N_u^2 + N_c^2)^{-0.5} [(N_u) T_u^* + (N_c) T_c^*], \quad (2.3.9)$$

and L_n proportional to σ_c^2 in (2.3.6), leading to the fourth statistic

$$T - STD = (\sigma_u^2 + \sigma_c^2)^{-0.5} [T_u^* \sigma_u + T_c^* \sigma_c]. \quad (2.3.10)$$

The simulation study involved four distributional forms for Z_i and generated data in such a way that uncensored and singly censored pairs were approximately 75% and 20% of the total sample size. For each case, 500 random samples of size 50 were generated.

The authors concluded that none of the statistics emerged as the best statistic for all distributional forms. For light-tailed distributions they recommended the statistics based on T-EQ for small to moderate sample sizes and WL Wilcoxon signed rank statistic if sample size is large.

Although not much difference in large sample powers was observed, Popovich and Rao (1985) mentioned that the selection of test procedure for testing H_0 should not only take into account the power of the test procedure, but also should be based on the availability of critical values and the level of difficulty involved in the implementation of the test procedures. As mentioned earlier, critical values of statistics $T_n(N_u, N_c)$ when sample size is small are easy to generate but that is not the case for WL Wilcoxon signed rank statistic. For large sample sizes, critical values for $T_n(N_u, N_c)$ are obtained by using the standard normal distribution.

Woolson and Lachenbruch (1980) claimed that the conditional null distribution of the statistics in the WL-class have asymptotic normal distribution as $\min(N_u, N_c) \rightarrow \infty$. Until Dabrowska (1990) established the asymptotic normality of T in (2.3.1), it was not clear if its asymptotic normality holds unconditionally as the overall sample size, $n \rightarrow \infty$.

For selecting the coefficients L_n , it was very natural to consider the possibility of selection based on the conditional Pitman Asymptotic Relative Efficiency (PARE) as $\min(n_u, n_c) \rightarrow \infty$. However Popovich and Rao (1985) noted that the expressions for PARE were complicated and they could not simplify them successfully.

Dabrowska (1990) represented the WL-class of statistics using score functions and counting processes in the form,

$$T = \int J_u(1 - \hat{S}(t)) d(N_1(t) - N_2(t)) + \int J_c(1 - \hat{S}(t)) d(N_3(t) - N_4(t)), \quad (2.3.11)$$

where $\hat{S}(t)$ denotes the Kaplan-Meier (1958) estimator of $P(|Z| > t)$ based on the $|Z_i|$, $i \in \cup_{j=1}^4 B_j$. Also, $J_u(\cdot)$ and $J_c(\cdot)$ are score functions defined on $[0, 1]$ such that,

$$J_u(v) = -\frac{d}{dv}\{(1 - v) J_c(v)\}. \quad (2.3.12)$$

Dabrowska's choice of score functions was motivated by the fact that the censored data version of the signed rank statistics considered by Woolson and Lachenbruch (1980) satisfied (2.3.12). For testing the null hypothesis of bivariate symmetry under log-linear models, (2.3.2), the score functions of Woolson and Lachenbruch (1980) are,

$$J_u(v) = -\frac{\phi'(w)}{\phi(w)} \quad \text{and} \quad J_c(v) = 2\frac{\phi(w)}{(1 - w)},$$

where $w = \Phi(\frac{1}{2} + \frac{v}{2})$ and Φ is the c.d.f. corresponding to ϕ .

As mentioned earlier, censored data versions of Sign and Wilcoxon signed rank statistics correspond to substituting double exponential and logistic density for ϕ respectively and these are the two cases explicitly considered by Woolson and Lachenbruch. Dabrowska (1990) also considered these two special cases of T . The corresponding scores are,

$$J_u(v) = J_c(v) = 1,$$

for T to be the same as WL Sign statistic and

$$J_u(v) = v \quad \text{and} \quad J_c(v) = \frac{1}{2} + \frac{v}{2},$$

for T to be asymptotically equivalent to WL Wilcoxon signed rank statistic.

Dabrowska used the representation at (2.3.11) to investigate the properties of WL signed rank statistics. She derived the asymptotic distributions of the statistics under H_0 and as well as under a sequence of contiguous alternatives and examined their Pitman asymptotic relative efficiencies using a log-linear model.

2.4 Objective

The general objective in this dissertation is to investigate the properties of the PR-class and WL-class of statistics with a view to develop statistics for tests of H_0 that are optimal with respect to appropriately chosen parametric alternatives. Following is a list of special tasks undertaken to achieve the above stated goals.

1. Use Dabrowska's (1990) representation of rank tests to derive the efficacies of members of the PR-class of statistics.
2. Determine the optimal statistics in the PR and WL-classes.
3. Compare the efficacies of the optimal statistics with the others using a log-linear model.
4. Develop methods for practical implementation of optimal tests.
5. Compare powers of optimal tests with others using simulation.

We assume throughout the remainder of this manuscript that the assumptions listed below are valid.

Assumptions :

- (AI). (X_{1i}, X_{2i}) , $i = 1, \dots, n$, are i.i.d. as a continuous bivariate random variable (X_1, X_2) with density $\psi(x_1, x_2)$.
- (AII). C_i , $i = 1, \dots, n$ are i.i.d. as a continuous random variable C and are independent of (X_{1i}, X_{2i}) , $i = 1, \dots, n$.
- (AIII). The probability of double censoring is strictly less than 1. That is,
 $p_0 = P(X_1 > C, X_2 > C) < 1$.

CHAPTER 3

EFFICACIES

3.1 Introduction

In this Chapter, we use counting process representations to study the properties of the PR-class of statistics. Asymptotic distributions of these statistics under the null hypothesis of bivariate symmetry are given in Section 3.2. In Section 3.3, the efficacies of the statistics in this class are derived using a contiguous sequence of parametric alternatives. Formulation of optimal statistic is done in Section 3.4. In Section 3.5, numerical comparison of the efficacies of a number of relevant statistics is done using a log-linear model.

3.2 Null Distribution of the Statistics in the PR-Class

Let $J(\cdot)$ be a score generating function defined on $[0, 1]$, and $\hat{F}_u(\cdot)$ be the conditional subsurvival function of $|Z_i|$ defined in Section 2.2.

Consider the statistic,

$$\begin{aligned} T = & \int L_{1u} J(1 - \hat{F}_u(t)) d(N_1(t) - N_2(t)) \\ & + \int L_{1c} d(N_3(t) - N_4(t)), \end{aligned} \quad (3.2.1)$$

where L_{1u} and L_{1c} are random variables satisfying the conditions,

(i) $L_{1u} = L_u + o_p(1)$, for some constant L_u and $0 \leq L_{1u} \leq 1$,

(ii) $L_{1c} = L_c + o_p(1)$, for some constant L_c and $0 \leq L_{1c} \leq 1$.

The form of T in (3.2.1) implies that, if $L_{1u} = 0$, then the uncensored pairs in the data have no contribution to the statistic. Similarly, if $L_{1c} = 0$, then the censored pairs in the data have no contribution to T . Indeed, T can be represented as a linear combination of two statistics:

$$T = L_{1u} T_{1u} + L_{1c} T_{1c}, \quad (3.2.2)$$

where

$$T_{1u} = \int J(1 - \hat{F}_u(t)) d(N_1(t) - N_2(t)), \quad (3.2.3)$$

and

$$\begin{aligned} T_{1c} &= \int d(N_3(t) - N_4(t)) \\ &= N_{c+} - N_{c-}. \end{aligned} \quad (3.2.4)$$

Note that T_{1c} is the Sign statistic based on Z_i in $B_3 \cup B_4$. Also, corresponding to the score function

$$J(1 - \hat{F}_u) = (1 - \hat{F}_u), \quad (3.2.5)$$

T_{1u} is the Wilcoxon signed rank statistic based on Z_i in $B_1 \cup B_2$, because

$$\begin{aligned} T_{1u} &= \int (1 - \hat{F}_u(t)) d(N_1(t) - N_2(t)) \\ &= \sum_{i \in B_1 \cup B_2} \Psi(Z_i) R_i^+ - \sum_{i \in B_1 \cup B_2} (1 - \Psi(Z_i)) R_i^+ \\ &= 2 \left(\sum_{i \in B_1 \cup B_2} \Psi(Z_i) R_i^+ - \frac{n_u(1 + n_u)}{4} \right) \end{aligned}$$

$$\begin{aligned}
&= 2 \left(\sum_{i \in B_1 \cup B_2} \Psi(Z_i) R_i^+ - \mu_u \right) \\
&= 2(T_u - \mu_u),
\end{aligned}$$

where T_u and μ_u are given in (2.3.3) and (2.3.6), respectively.

The following two Lemmas will be used in the proof of the asymptotic normality of T .

Lemma 3.2.1 Suppose the score generating function J is continuous and

- (i) there exists $M < \infty$ such that $|J(v)| < M$ for all $0 \leq v \leq 1$,
- (ii) there exists $M_0 < \infty$ such that $|J'(v)| < M_0$ for all $0 \leq v \leq 1$.

Let T be as in (3.2.1) and define

$$T' = \int L_{1u} J(1 - F_u(t)) d(N_1(t) - N_2(t)) + \int L_{1c} d(N_3(t) - N_4(t)).$$

Then

$$\frac{1}{\sqrt{n}}(T - T') = o_p(1).$$

Proof. Notice that, if $p_u = 0$, then

$$(T - T') = 0 \quad \text{with probability 1.}$$

For $p_u > 0$, we have

$$\begin{aligned}
&\frac{1}{\sqrt{n}}(T - T') \\
&= \frac{1}{\sqrt{n}} \left[\int L_{1u} J(1 - \hat{F}_u(t)) d(N_1(t) - N_2(t)) \right. \\
&\quad \left. - \int L_{1u} J(1 - F_u(t)) d(N_1(t) - N_2(t)) \right] \\
&= \frac{1}{\sqrt{n}} \int L_{1u} [J(1 - \hat{F}_u(t)) - J(1 - F_u(t))] d(N_1(t) - N_2(t)). \quad (3.2.6)
\end{aligned}$$

Taylor series expansion of $J(\cdot)$ around $(1 - F_u(t))$ gives,

$$J(1 - \hat{F}_u(t)) = J(1 - F_u(t)) + (F_u(t) - \hat{F}_u(t))J'(\xi(t)), \quad (3.2.7)$$

where

$$\min(1 - \hat{F}_u(t), 1 - F_u(t)) < \xi(t) < \max(1 - \hat{F}_u(t), 1 - F_u(t)).$$

Using (3.2.7) and boundedness of J in (3.2.6) we have

$$\begin{aligned} \left| \frac{1}{\sqrt{n}}(T - T') \right| &= \left| \frac{1}{\sqrt{n}} \int L_{1u}(F_u(t) - \hat{F}_u(t)) J'(\xi(t)) d(N_1(t) - N_2(t)) \right| \\ &\leq \frac{1}{\sqrt{n}} M_o \sup |F_u(t) - \hat{F}_u(t)| \sup |L_{1u}| \left| \int d(N_1(t) - N_2(t)) \right|. \end{aligned} \quad (3.2.8)$$

Now, using the fact that, for $p_u > 0$,

$$\frac{1}{\hat{p}_u} - \frac{1}{p_u} = o_p(1),$$

and

$$\sup |F_u^*(t) - \hat{F}_u^*(t)| = o_p(1),$$

we have

$$\begin{aligned} \sup |F_u(t) - \hat{F}_u(t)| &= \sup \left| \frac{F_u^*(t)}{p_u} - \frac{\hat{F}_u^*(t)}{\hat{p}_u} \right| \\ &= \sup \left| F_u^*(t) \left(\frac{1}{p_u} - \frac{1}{\hat{p}_u} \right) + \frac{1}{\hat{p}_u} (F_u^*(t) - \hat{F}_u^*(t)) \right| \\ &\leq \left| \frac{1}{p_u} - \frac{1}{\hat{p}_u} \right| + \frac{1}{\hat{p}_u} \sup |F_u^*(t) - \hat{F}_u^*(t)| \\ &= o_p(1) + O_p(1) o_p(1) \\ &= o_p(1) + o_p(1) \\ &= o_p(1). \end{aligned} \quad (3.2.9)$$

Again,

$$\begin{aligned}
\frac{1}{\sqrt{n}} \left| \int d(N_1(t) - N_2(t)) \right| &\leq \frac{1}{\sqrt{n}} \int d(N_1(t) + N_2(t)) \\
&= \sqrt{n} \hat{p}_u \\
&= O_p(1),
\end{aligned} \tag{3.2.10}$$

and

$$\sup |L_{1u}| \leq 1. \tag{3.2.11}$$

Therefore, using (3.2.9), (3.2.10) and (3.2.11) in (3.2.8), we get

$$\frac{1}{\sqrt{n}}(T - T') = o_p(1).$$

Lemma 3.2.2 Suppose the conditions of Lemma 3.2.1 are satisfied. Let

$$T_1 = \int L_u J(1 - F_u(t)) d(N_1(t) - N_2(t)) + \int L_c d(N_3(t) - N_4(t)).$$

Then for T' defined in Lemma 3.2.1,

$$\frac{1}{\sqrt{n}}(T' - T_1) = o_p(1).$$

Proof. We have

$$\begin{aligned}
&\left| \frac{1}{\sqrt{n}}(T' - T_1) \right| \\
&= \left| \frac{1}{\sqrt{n}} \int (L_u - L_{1u}) J(1 - F_u(t)) d(N_1(t) - N_2(t)) \right. \\
&\quad \left. + \int (L_c - L_{1c}) d(N_3(t) - N_4(t)) \right| \\
&\leq o_p(1) \sup |J(1 - F_u(t))| \left| \frac{1}{\sqrt{n}} \int d(N_1(t) - N_2(t)) \right| \\
&\quad + o_p(1) \frac{1}{\sqrt{n}} \left| \int d(N_3(t) - N_4(t)) \right|.
\end{aligned}$$

Now, using the fact that

$$\begin{aligned} \frac{1}{\sqrt{n}} \left| \int d(N_3(t) - N_4(t)) \right| &\leq \frac{1}{\sqrt{n}} \int d(N_3(t) + N_4(t)) \\ &= \frac{N_c}{\sqrt{n}} \\ &= O_p(1), \end{aligned}$$

we get

$$\begin{aligned} \left| \frac{1}{\sqrt{n}}(T' - T_1) \right| &= o_p(1)O_p(1) + o_p(1)O_p(1) \\ &= o_p(1). \end{aligned}$$

The following theorem gives the asymptotic null distribution of T in (3.2.1).

Theorem 3.2.1 Assume AI–AIII in Section 2.4 are satisfied. Suppose that the conditions on the score generating function J given in Lemma 3.2.1 are valid. Then under the hypothesis, H_0 , that (X_1, X_2) has a distribution symmetric about 0, T/\sqrt{n} converges weakly to a normal distribution with mean

$$\mu_T = 0$$

and variance

$$\sigma_T^2 = 2 \left[\int L_u^2 J^2(1 - F_u(t)) f_1(t) dt + \int L_c^2 f_3(t) dt \right].$$

A consistent estimator of σ_T^2 is given by

$$\hat{\sigma}_T^2 = \frac{1}{n} \left[\sum_{i=1}^2 L_u^2 \int J^2(1 - \hat{F}_u(t)) dN_i(t) + \sum_{i=3}^4 L_c^2 \int dN_i(t) \right].$$

Proof. In order to prove the asymptotic normality of T , note that

$$T = T_1 + (T - T') + (T' - T_1),$$

where,

$$T_1 = \int L_u J(1 - F_u(t))d(N_1(t) - N_2(t)) + \int L_c d(N_3(t) - N_4(t)),$$

and

$$T' = \int L_{1u} J(1 - F_u(t))d(N_1(t) - N_2(t)) + \int L_{1c} d(N_3(t) - N_4(t)).$$

From Lemma 3.1.1 and Lemma 3.1.2 we have

$$\frac{1}{\sqrt{n}}(T - T') = o_p(1),$$

and

$$\frac{1}{\sqrt{n}}(T' - T_1) = o_p(1).$$

Therefore, a proof of the theorem can be given by showing that T_1/\sqrt{n} is asymptotically normal with mean 0 and variance σ_T^2 . Now, T_1 can be expressed as :

$$\begin{aligned} T_1 &= \sum_{i=1}^n \epsilon_i [\delta_i L_u J(1 - F_u(|Z_i|)) + L_c (1 - \delta_i)] \\ &= \sum_{i=1}^n \beta_i, \end{aligned} \tag{3.2.12}$$

where

$$\beta_i = \epsilon_i [\delta_i L_u J(1 - F_u(|Z_i|)) + L_c (1 - \delta_i)],$$

and

$$\delta_i = \delta_{1i} \delta_{2i}, \quad i = 1, \dots, n.$$

Note that, if $\delta_i = 1$, then both observations in the i th pair are uncensored. Under H_0 , and conditional on δ_i and $|Z_i|$, the probability of $(Z_i > 0)$ is same as the probability of $(Z_i < 0)$. Therefore,

$$E[\epsilon_i \mid \delta_i, |Z_i|] = 0, \tag{3.2.13}$$

so that

$$\begin{aligned}
 E(\beta_i) &= E[E(\beta_i | \delta_i, |Z_i|)] \\
 &= E \left[E(\epsilon_i | \delta_i, |Z_i|) [\delta_i L_u J(1 - F_u(|Z_i|)) + L_c (1 - \delta_i)] \right] \\
 &= 0.
 \end{aligned}$$

Also,

$$\begin{aligned}
 var(\beta_i) &= E(\beta_i^2) \\
 &= E \left[\epsilon_i^2 \left(\delta_i^2 L_u^2 J^2(1 - F_u(|Z_i|)) + L_c^2 (1 - \delta_i)^2 \right. \right. \\
 &\quad \left. \left. + 2\delta_i(1 - \delta_i) L_u L_c J(1 - F_u(|Z_i|)) \right) \right] \\
 &= E E \left[\left(\delta_i^2 L_u^2 J^2(1 - F_u(|Z_i|)) + L_c^2 (1 - \delta_i)^2 \right. \right. \\
 &\quad \left. \left. + 2 L_u L_c \delta_i(1 - \delta_i) J(1 - F_u(|Z_i|)) \right) | \delta_i \right] \\
 &= P[\delta_i = 1] E[L_u^2 J^2(1 - F_u(|Z_i|)) | \delta_i = 1] + P[\delta_i = 0] E[L_c^2 | \delta_i = 0] \\
 &= 2F_1(0) \int L_u^2 J^2(1 - F_u(t)) dF_{t|\delta_i=1}(t) + 2F_3(0) \int L_c^2 dF_{t|\delta_i=0}(t) \\
 &= 2F_1(0) \int L_u^2 J^2(1 - F_u(t)) \frac{2f_1(t)}{2F_1(0)} dt + 2F_3(0) \int L_c^2 \frac{2f_3(t)}{2F_3(0)} dt \\
 &= 2 \left[\int L_u^2 J^2(1 - F_u(t)) f_1(t) dt + \int L_c^2 f_3(t) dt \right] \\
 &= \sigma_T^2.
 \end{aligned}$$

Therefore, since the β_i are independent,

$$var \left(\frac{T_1}{\sqrt{n}} \right) = \sigma_T^2.$$

Since T_1 is a sum of *i.i.d* random variables with mean zero and variance σ_T^2 ($\sigma_T^2 < \infty$), the asymptotic normality of T_1/\sqrt{n} follows from Lindeberg-Lévy central limit theorem.

Next, we proceed to prove that $\hat{\sigma}_T^2$ is consistent for σ_T^2 . Observe that,

$$\hat{\sigma}_T^2 = \tilde{\sigma}_T^2 + (\hat{\sigma}_T^2 - \tilde{\sigma}_T^2),$$

where

$$\tilde{\sigma}_T^2 = \frac{1}{n} \left[\sum_{i=1}^2 \int L_u^2 J^2(1 - F_u(t)) dN_i(t) + \sum_{i=3}^4 L_c^2 dN_i(t) \right]. \quad (3.2.14)$$

Consistency of $\hat{\sigma}_T^2$ follows if we show that $\tilde{\sigma}_T^2$ is consistent for σ_T^2 and

$$\hat{\sigma}_T^2 - \tilde{\sigma}_T^2 = o_p(1).$$

From (3.2.14), we have

$$\begin{aligned} \tilde{\sigma}_T^2 &= \frac{1}{n} \left[\sum_{i=1}^n [\delta_i L_u^2 J^2(1 - F_u(|Z_i|)) + L_c^2 (1 - \delta_i)] \right] \\ &= \frac{1}{n} \sum_{i=1}^n \alpha_i, \end{aligned}$$

where

$$\alpha_i = \delta_i L_u^2 J^2(1 - F(|Z_i|)) + L_c^2 (1 - \delta_i) \quad i = 1, \dots, n,$$

are i.i.d. random variables with mean,

$$\begin{aligned} E(\alpha_i) &= E \left[E(\alpha_i \mid \delta_i) \right] \\ &= E E \left[\delta_i L_u^2 J^2(1 - F_u(|Z_i|)) + L_c^2 (1 - \delta_i) \mid \delta_i \right] \\ &= P[\delta_i = 1] E[L_u^2 J^2(1 - F_u(|Z_i|)) \mid \delta_i = 1] + P[\delta_i = 0] E[L_c^2 \mid \delta_i = 0] \\ &= 2F_1(0) \int L_u^2 J^2(1 - F_u(t)) dF_{t|\delta_i=1}(t) + 2F_3(0) \int L_c^2 dF_{t|\delta_i=0}(t) \\ &= 2F_1(0) \int L_u^2 J^2(1 - F_u(t)) \frac{2f_1(t)}{2F_1(0)} dt + 2F_3(0) \int L_c^2 \frac{2f_3(t)}{2F_3(0)} dt \\ &= 2 \left[\int L_u^2 J^2(1 - F_u(t)) f_1(t) dt + \int L_c^2 f_3(t) dt \right] \\ &= \sigma_T^2. \end{aligned}$$

Therefore,

$$\begin{aligned} E(\tilde{\sigma}_T^2) &= 2 \left[\int L_u^2 J^2(1 - F_u(t)) f_1(t) dt + \int L_c^2 f_3(t) dt \right] \\ &= \sigma_T^2. \end{aligned} \quad (3.2.15)$$

Since $\tilde{\sigma}_T^2$ is a sum of i.i.d. random variables with mean σ_T^2 ($\sigma_T^2 < \infty$), consistency of $\tilde{\sigma}_T^2$ follows from the law of large numbers.

Now, we have to prove that

$$\tilde{\sigma}_T^2 - \hat{\sigma}_T^2 = o_p(1).$$

We can write,

$$\tilde{\sigma}_T^2 - \hat{\sigma}_T^2 = \frac{1}{n} \sum_{i=1}^2 \int L_u^2 \left[J^2(1 - F_u(t)) - J^2(1 - \hat{F}_u(t)) \right] dN_i(t).$$

Applying Taylor series expansion, we obtain,

$$J^2(1 - \hat{F}_u(t)) = J^2(1 - F_u(t)) + (F_u(t) - \hat{F}_u(t)) 2 J(\xi(t)) J'(\xi(t))$$

where

$$\min(1 - \hat{F}_u(t), 1 - F_u(t)) < \xi(t) < \max(1 - \hat{F}_u(t), 1 - F_u(t)).$$

Therefore,

$$\begin{aligned} |\tilde{\sigma}_T^2 - \hat{\sigma}_T^2| &= \left| \frac{1}{n} \sum_{i=1}^2 \int L_u^2 (F_u(t) - \hat{F}_u(t)) 2 J(\xi(t)) J'(\xi(t)) dN_i(t) \right| \\ &\leq \frac{1}{n} M M_0 L_u^2 \sup |F_u(t) - \hat{F}_u(t)| \left| \sum_{i=1}^2 \int dN_i(t) \right|. \end{aligned}$$

Since,

$$\frac{1}{n} \sum_{i=1}^2 \int dN_i(t) = \frac{N_u + N_c}{n} = O_p(1),$$

using the same argument as in the proof of Lemma 3.2.1, we have

$$\tilde{\sigma}_T^2 - \hat{\sigma}_T^2 = o_p(1).$$

This completes the proof of the Theorem 3.2.1.

3.3 Efficacies of the Statistics in the PR-Class

In order to derive the efficacies of the tests based on statistics of the form T in (3.2.1), we consider the sequence of contiguous alternatives with densities of the form,

$$\psi_n(x_1, x_2) = \psi(x_1, x_2) \left\{ 1 + \frac{\gamma_n(x_1, x_2)}{\sqrt{n}} \right\}, \quad (3.3.1)$$

where $\{\gamma_n(x_1, x_2)\}$ is a sequence of asymmetric functions converging to an asymmetric function $\gamma(x_1, x_2)$ for almost all (x_1, x_2) such that

$$\int \gamma_n(x_1, x_2) \psi_n(x_1, x_2) dx_1 dx_2 = \int \gamma(x_1, x_2) \psi(x_1, x_2) dx_1 dx_2 = 0.$$

Dabrowska (1990) considered the same sequence of contiguous alternatives in her derivation of the efficacies of the WL-class of statistics.

In what follows, a parametric sequence of alternatives to H_0 with real parameter, θ , will be specified as $\{\psi(x_1, x_2, : \theta_n)\}$ where $\theta_n = \theta_0 + \frac{c}{\sqrt{n}}$ for some constant $c > 0$, and the density under the null hypothesis is a symmetric density, $\psi(x_1, x_2, : \theta_0)$. Then $\gamma(x_1, x_2)$ is given by

$$\gamma(x_1, x_2) = c \frac{d}{d\theta} \log \psi(x_1, x_2 : \theta) \Big|_{\theta=\theta_0}.$$

Without loss of generality we will assume $c = 1$ because in the expression of efficacy, c appears in the numerator and denominator and therefore efficacy is invariant with respect to the value of c .

In order to derive the efficacy of a statistic in the PR-class, the distribution of T in (3.2.1), under alternative is required. By applying Le Cam's third lemma (Hájek and Sidák, 1967) we can find the asymptotic distribution of T under contiguous alternatives. Following definitions will be used in the derivation of the asymptotic

distribution of T .

$$\begin{aligned}
A_0 &= p_0^{-1} \int \left\{ \int_u^\infty \int_u^\infty \gamma(x_1, x_2) \psi(x_1, x_2) dx_1 dx_2 \right\} dG(u), \\
A_1(t) &= [f_1(t)]^{-1} \int \overline{G}(u) \gamma(u-t, u) \psi(u-t, u) du, \\
A_2(t) &= [f_2(t)]^{-1} \int \overline{G}(u) \gamma(u, u-t) \psi(u, u-t) du, \\
A_3(t) &= [f_3(t)]^{-1} \int \left\{ \int \gamma(u-t, y) \psi(u-t, y) dy \right\} dG(u),
\end{aligned}$$

and

$$A_4(t) = [f_4(t)]^{-1} \int \left\{ \int \gamma(y, u-t) \psi(y, u-t) dy \right\} dG(u). \quad (3.3.2)$$

Let $f_{j\theta}(\cdot)$, $j = 1, 2, 3, 4$, and $p_{0\theta}$ denote, respectively, the improper densities and the probability of double censoring obtained by replacing $\psi(x_1, x_2)$ with $\psi(x_1, x_2 : \theta)$ in (2.2.4) and (2.2.3). Then, it is easy to see that

$$A_0 = \frac{d}{d\theta} \log p_{0\theta} \Big|_{\theta = \theta_0},$$

and

$$A_j(t) = \frac{d}{d\theta} \log f_{j\theta}(t) \Big|_{\theta = \theta_0}, \quad j = 1, 2, 3, 4.$$

For example,

$$\begin{aligned}
A_0 &= p_{0\theta_0}^{-1} \int \left\{ \int_u^\infty \int_u^\infty \frac{d \log \psi(x_1, x_2 : \theta)}{d\theta} \Big|_{\theta=\theta_0} \psi(x_1, x_2 : \theta_0) dx_1 dx_2 \right\} dG(u) \\
&= p_{0\theta_0}^{-1} \int \left\{ \int_u^\infty \int_u^\infty \frac{\psi'(x_1, x_2 : \theta)}{\psi(x_1, x_2 : \theta)} \Big|_{\theta=\theta_0} \psi(x_1, x_2 : \theta_0) dx_1 dx_2 \right\} dG(u) \\
&= p_{0\theta_0}^{-1} \int \left\{ \int_u^\infty \int_u^\infty \psi'(x_1, x_2 : \theta) dx_1 dx_2 \right\} \Big|_{\theta=\theta_0} dG(u) \\
&= \frac{d}{d\theta} \left[p_{0\theta_0}^{-1} \int \left\{ \int_u^\infty \int_u^\infty \psi(x_1, x_2 : \theta) dx_1 dx_2 \right\} \Big|_{\theta=\theta_0} dG(u) \right] \\
&= \frac{d \log p_{0\theta}}{d\theta} \Big|_{\theta=\theta_0}.
\end{aligned}$$

We also define $f_{jn}(\cdot)$, $j = 1, 2, 3, 4$, and p_{0n} just as $f_j(\cdot)$ and p_0 , except that $\psi(x_1, x_2)$ is replaced by $\psi_n(x_1, x_2)$, as given in (3.3.1). The following theorem gives

the joint distribution of T in (3.2.1) and the log-likelihood ratio :

$$\log \frac{dP_n}{dP} = \sum_{j=1}^4 \int \log \frac{f_{jn}(t)}{f_j(t)} dN_j(t) + \log \frac{p_{0n}}{p_0} \sum_{i=1}^n \zeta_i, \quad (3.3.3)$$

where $\zeta_i = (1 - \delta_{1i})(1 - \delta_{2i})$.

Theorem 3.3.1 Suppose that the assumptions of Theorem 3.2.1 are satisfied and that, as $n \rightarrow \infty$,

$$A_{0n} = 2\sqrt{n} \left[\left(\frac{p_{0n}}{p_0} \right)^{1/2} - 1 \right] \rightarrow A_0,$$

and

$$\int (A_{jn}(t) - A_j(t))^2 f_j(t) dt \rightarrow 0,$$

where

$$A_{jn}(t) = 2\sqrt{n} \left[\left(\frac{f_{jn}(t)}{f_j(t)} \right)^{1/2} - 1 \right], \quad j = 1, 2, 3, 4.$$

Then, under the null hypothesis, $(\log \frac{dP_n}{dP}, \frac{T}{\sqrt{n}})$ converges weakly to a normal distribution with mean $(-\sigma_0^2/2, 0)$ and covariance matrix

$$\begin{pmatrix} \sigma_0^2 & C_T \\ C_T & \sigma_T^2 \end{pmatrix},$$

where σ_T^2 is defined in Theorem 3.2.1 and

$$\begin{aligned} \sigma_0^2 &= \int (A_1^2(t) + A_2^2(t)) f_1(t) dt + \int (A_3^2(t) + A_4^2(t)) f_3(t) dt + A_0^2 p_0^2, \\ C_T &= \int L_u J(1 - F(t))(A_1(t) - A_2(t)) dt + \int L_c (A_3(t) - A_4(t)) dt. \end{aligned}$$

Proof. To derive the joint distribution of $\log \frac{dP_n}{dP}$ and T/\sqrt{n} , note that, by Le Cam's second lemma (Hájek and Sidák, 1967), it is enough to consider the joint distribution of T/\sqrt{n} and K_n , where

$$K_n = \frac{1}{\sqrt{n}} \left[\sum_{j=1}^4 \int A_{jn}(t) dN_j(t) + A_{0n} \sum_{i=1}^n \zeta_i \right]. \quad (3.3.4)$$

From Theorem 3.2.1, we know that asymptotic distribution of T is the same as the asymptotic distribution of T_1 defined in Lemma 3.2.2. Therefore, we will consider the joint distribution of T_1/\sqrt{n} and K_n . However, to derive the distribution of K_n , it is convenient to consider,

$$S_n = \frac{1}{\sqrt{n}} \left[\sum_{j=1}^4 \int A_j(t) dN_j(t) + A_0 \sum_{i=1}^n \zeta_i \right]. \quad (3.3.5)$$

Now, we proceed to derive the expectation of S_n . We have

$$\begin{aligned} \sqrt{n} S_n &= \sum_{j=1}^4 \int A_j(t) dN_j(t) + A_0 \sum_{i=1}^n \zeta_i \\ &= \sum_{i=1}^n \left[\delta_{1i} \delta_{2i} \left[\frac{(1+\epsilon_i)}{2} A_1(|Z_i|) + \frac{(1-\epsilon_i)}{2} A_2(|Z_i|) \right] \right. \\ &\quad \left. + (\delta_{1i}(1-\delta_{2i}) + (1-\delta_{1i})\delta_{2i}) \left[\frac{(1+\epsilon_i)}{2} A_3(|Z_i|) + \frac{(1-\epsilon_i)}{2} A_4(|Z_i|) \right] \right. \\ &\quad \left. + A_0(1-\delta_{1i})(1-\delta_{2i}) \right] \\ &= \sum_{i=1}^n \beta_i^*. \end{aligned} \quad (3.3.6)$$

Clearly, β_i^* 's are i.i.d. random variables. Under H_0 , the mean of β_i^* is,

$$\begin{aligned} E(\beta_i^*) &= E E[\beta_i^* \mid \delta_{1i} \delta_{2i}] \\ &= P[\delta_{1i} = \delta_{2i} = 1] E \left[\frac{(1+\epsilon_i)}{2} A_1(|Z_i|) + \frac{(1-\epsilon_i)}{2} A_2(|Z_i|) \mid \delta_{1i} = \delta_{2i} = 1 \right] \\ &\quad + P[\delta_{1i} = 1, \delta_{2i} = 0] E \left[\frac{(1+\epsilon_i)}{2} A_3(|Z_i|) \right. \\ &\quad \left. + \frac{(1-\epsilon_i)}{2} A_4(|Z_i|) \mid \delta_{1i} = 1, \delta_{2i} = 0 \right] \\ &\quad + P[\delta_{1i} = 0, \delta_{2i} = 1] E \left[\frac{(1+\epsilon_i)}{2} A_3(|Z_i|) \right. \\ &\quad \left. + \frac{(1-\epsilon_i)}{2} A_4(|Z_i|) \mid \delta_{1i} = 0, \delta_{2i} = 1 \right] \\ &\quad + P[\delta_{1i} = \delta_{2i} = 0] E [A_0 \mid \delta_{1i} = \delta_{2i} = 0]. \end{aligned} \quad (3.3.7)$$

Under H_0 , $F_1(0) = F_2(0)$ and $P[\delta_{1i} = \delta_{2i} = 1] = 2F_1(0)$. Therefore the first term on the right hand side of (3.3.7) simplifies to,

$$\begin{aligned}
& 2F_1(0) E \left[\frac{(1 + \epsilon_i)}{2} A_1(|Z_i|) + \frac{(1 - \epsilon_i)}{2} A_2(|Z_i|) \mid \delta_{1i} = \delta_{2i} = 1 \right] \\
&= 2F_1(0) P[\epsilon_i = 1 \mid \delta_{1i} = \delta_{2i} = 1, \epsilon_i = 1] E[A_1(|Z_i|) \mid \delta_{1i} = \delta_{2i} = 1, \epsilon_i = 1] \\
&\quad + 2F_2(0) P[\epsilon_i = -1 \mid \delta_{1i} = \delta_{2i} = 1, \epsilon_i = -1] \times \\
&\quad E[A_2(|Z_i|) \mid \delta_{1i} = \delta_{2i} = 1, \epsilon_i = -1] \\
&= 2F_1(0) \frac{1}{2} \int A_1(t) \frac{f_1(t)}{F_1(0)} dt + 2F_2(0) \frac{1}{2} \int A_2(t) \frac{f_2(t)}{F_2(0)} dt \\
&= \int A_1(t) f_1(t) dt + \int A_2(t) f_2(t) dt.
\end{aligned}$$

Similar calculations can be done for other terms and we can write,

$$\begin{aligned}
E(\beta_i^*) &= \int A_1(t) f_1(t) dt + \int A_2(t) f_2(t) dt \\
&\quad + \int A_3(t) f_3(t) dt + \int A_4(t) f_4(t) dt + A_0 p_0.
\end{aligned}$$

After some simplification, it can be shown that

$$E(\beta_i^*) = 0. \tag{3.3.8}$$

Now consider,

$$\begin{aligned}
E(\beta_i^{*2}) &= E E[\beta_i^{*2} \mid \delta_{1i} \delta_{2i}] \\
&= P[\delta_{1i} = \delta_{2i} = 1] E \left[\left(\frac{(1 + \epsilon_i)}{2} A_1(|Z_i|) \right. \right. \\
&\quad \left. \left. + \frac{(1 - \epsilon_i)}{2} A_2(|Z_i|) \right)^2 \mid \delta_{1i} = \delta_{2i} = 1 \right]
\end{aligned}$$

$$\begin{aligned}
& + P[\delta_{1i} = 1, \delta_{2i} = 0] E \left[\left(\frac{(1 + \epsilon_i)}{2} A_3(|Z_i|) \right. \right. \\
& \quad \left. \left. + \frac{(1 - \epsilon_i)}{2} A_4(|Z_i|) \right)^2 \mid \delta_{1i} = 1, \delta_{2i} = 0 \right] \\
& + P[\delta_{1i} = 0, \delta_{2i} = 1] E \left[\left(\frac{(1 + \epsilon_i)}{2} A_3(|Z_i|) \right. \right. \\
& \quad \left. \left. + \frac{(1 - \epsilon_i)}{2} A_4(|Z_i|) \right)^2 \mid \delta_{1i} = 0, \delta_{2i} = 1 \right] \\
& + P[\delta_{1i} = \delta_{2i} = 0] E \left[A_0^2 \mid \delta_{1i} = \delta_{2i} = 0 \right]. \tag{3.3.9}
\end{aligned}$$

Under the null hypothesis, the first term on the right hand side of (3.3.9) equals

$$\begin{aligned}
& 2F_1(0) E \left[\left(\frac{(1 + \epsilon_i)}{2} A_1(|Z_i|) + \frac{(1 - \epsilon_i)}{2} A_2(|Z_i|) \right)^2 \mid \delta_{1i} = \delta_{2i} = 1 \right] \\
& = 2F_1(0) P(\epsilon_i = 1 \mid \delta_{1i} = \delta_{2i} = 1) E \left[A_1^2(|Z_i|) \mid \delta_{1i} = \delta_{2i} = 1, \epsilon_i = 1 \right] \\
& \quad + 2F_2(0) P(\epsilon_i = -1 \mid \delta_{1i} = \delta_{2i} = 1) E \left[A_2^2(|Z_i|) \mid \delta_{1i} = \delta_{2i} = 1, \epsilon_i = -1 \right] \\
& = 2F_1(0) \frac{1}{2} \int A_1^2(t) \frac{f_1(t)}{F_1(0)} dt + 2F_2(0) \frac{1}{2} \int A_2^2(t) \frac{f_2(t)}{F_2(0)} dt.
\end{aligned}$$

Similar calculations can be done for other terms in (3.3.9) to obtain

$$\begin{aligned}
E(\beta_i^{*2}) & = 2F_1(0) \frac{1}{2} \int A_1^2(t) \frac{f_1(t)}{F_1(0)} dt + 2F_2(0) \frac{1}{2} \int A_2^2(t) \frac{f_2(t)}{F_2(0)} dt \\
& \quad + 2F_3(0) \frac{1}{2} \int A_3^2(t) \frac{f_3(t)}{F_3(0)} dt + 2F_4(0) \frac{1}{2} \int A_4^2(t) \frac{f_4(t)}{F_4(0)} dt + p_0 A_0^2 \\
& = \int (A_1^2(t) + A_2^2(t)) f_1(t) dt + \int (A_3^2(t) + A_4^2(t)) f_3 dt + p_0 A_0^2 \\
& = \sigma_0^2.
\end{aligned}$$

Since β_i^* 's are i.i.d.,

$$\text{var}(\sqrt{n}S_n) = n \text{var}(\beta_i^*).$$

That is,

$$\begin{aligned} \text{var}(S_n) &= \text{var}(\beta_i^*) \\ &= E(\beta_i^{*2}) \\ &= \sigma_0^2. \end{aligned}$$

Therefore, under H_0 , S_n is a sum of i.i.d. random variables with mean zero and variance σ_0^2 , so that by the central limit theorem,

$$\frac{1}{\sqrt{n}} S_n \sim \text{Asymptotically Normal} \left(0, \frac{\sigma_0^2}{n} \right).$$

Now, the next step is to find $E(K_n)$. By definition,

$$\begin{aligned} f_{1n}(t) &= \int \psi_n(u-t, t) \bar{G}(u) du \\ &= \int \psi(u-t, t) \bar{G}(u) du + \frac{1}{\sqrt{n}} \int \gamma_n(u-t, t) \psi(u-t, t) \bar{G}(u) du \\ &= f_1(t) + \frac{1}{\sqrt{n}} \int \gamma(u-t, t) \psi(u-t, t) \bar{G}(u) du \\ &= f_1(t) + h_n(t), \quad \text{say.} \end{aligned} \tag{3.3.10}$$

Let

$$g(u) = (f_1(t) + u)^{\frac{1}{2}}.$$

Differentiating $g(u)$ with respect to u ,

$$\begin{aligned} g'(u) &= \frac{1}{2} \frac{1}{\sqrt{f_1(t) + u}}, \\ g''(u) &= -\frac{1}{4} \frac{1}{(\sqrt{f_1(t) + u})^3}. \end{aligned}$$

Expanding $g(u)$ around $u = 0$, we get,

$$g(u) = g(0) + u g'(0) + \frac{u^2}{2!} g''(0) + o(u^2).$$

Then using (3.3.10) and the fact that $g(h_n(t)) = \sqrt{f_{1n}(t)}$ we get

$$\begin{aligned}\sqrt{f_{1n}(t)} &= \sqrt{f_1(t)} + \frac{1}{2\sqrt{f_1(t)}\sqrt{n}} \int \gamma(u-t, t) \psi(u-t, t) \overline{G}(u) du \\ &\quad + \frac{1}{8\sqrt{f_1(t)}f_1(t)n} \left[\int \gamma(u-t, t) \psi(u-t, t) \overline{G}(u) du \right]^2 + o(h_n^2(t)) \\ &= \sqrt{f_1(t)} + \frac{1}{2\sqrt{n}} \sqrt{f_1(t)} A_1(t) - \frac{1}{8n} \sqrt{f_1(t)} A_1^2(t) + o(h_n^2(t)).\end{aligned}$$

Therefore, using the definition of $A_{1n}(t)$ from the statement of Theorem 3.3.1,

$$\begin{aligned}A_{1n}(t) &= 2\sqrt{n} \left[\left(\frac{f_{1n}(t)}{f_1(t)} \right)^{1/2} - 1 \right] \\ &= A_1(t) - \frac{A_1^2(t)}{4\sqrt{n}} + o(n^{-\frac{1}{2}}).\end{aligned}$$

Similarly, it can be shown that,

$$A_{jn}(t) = A_j(t) - \frac{A_j^2(t)}{4\sqrt{n}} + o(n^{-\frac{1}{2}}), \quad j = 2, 3, 4, \quad (3.3.11)$$

and

$$A_{0n}(t) = A_0 - \frac{A_0^2}{4\sqrt{n}} + o(n^{-\frac{1}{2}}). \quad (3.3.12)$$

Following the technique used in the derivation of expectation of S_n , (3.3.11) and (3.3.12) we have

$$\begin{aligned}E(K_n) &= \sqrt{n} \left[\sum_{j=1}^4 \int A_j(t) f_j(t) dt + A_0 p_0 \right] \\ &\quad - \frac{1}{4} \left[\sum_{j=1}^4 \int A_j^2(t) f_j(t) dt + A_0^2 p_0 \right] + o(1) \\ &= -\frac{1}{4} \sum_{j=1}^4 \int A_{jn}^2(t) f_j(t) dt - \frac{1}{4} A_{0n}^2 p_0 \\ &\rightarrow -\frac{1}{4} \sigma_0^2.\end{aligned}$$

Now notice that

$$\begin{aligned}
\sqrt{n}(K_n - S_n) &= \sum_{i=1}^n \left[\delta_{1i} \delta_{2i} \left[\frac{(1 + \epsilon_i)}{2} (A_{1n}(|Z_i|) - A_1(|Z_i|)) \right. \right. \\
&\quad \left. \left. + \frac{(1 - \epsilon_i)}{2} (A_{2n}(|Z_i|) - A_2(|Z_i|)) \right] \right. \\
&\quad \left. + (\delta_{1i} (1 - \delta_{2i}) + (1 - \delta_{1i}) \delta_{2i}) \left[\frac{(1 + \epsilon_i)}{2} (A_{3n}(|Z_i|) - A_3(|Z_i|)) \right. \right. \\
&\quad \left. \left. + \frac{(1 - \epsilon_i)}{2} (A_{4n}(|Z_i|) - A_4(|Z_i|)) \right] \right. \\
&\quad \left. + A_0 (1 - \delta_{1i}) (1 - \delta_{2i}) \right] \\
&= \sum_{i=1}^n \beta'_i.
\end{aligned}$$

Following the technique used in the derivation of $E(\beta_i^{*2})$ we can show that

$$\begin{aligned}
\text{var}(\beta'_i) &\leq E(\beta_i'^2) \\
&= \sum_{j=1}^4 \int (A_{jn} - A_j)^2(t) f_j(t) dt + (A_{0n} - A_0)^2 p_0. \quad (3.3.13)
\end{aligned}$$

From (3.3.13), it can be shown that,

$$\text{var}(K_n - S_n) \leq \sum_{j=1}^4 \int (A_{jn} - A_j)^2(t) f_j(t) dt + (A_{0n} - A_0)^2 p_0 \rightarrow 0.$$

Therefore, under the null hypothesis, K_n is asymptotically normal with mean $-\frac{\sigma_0^2}{4}$ and variance σ_0^2 . We can use Le Cam's second lemma to complete the proof of the asymptotic normality of the log-likelihood $\log \frac{dP_n}{dP}$, that is

$$\log \frac{dP_n}{dP} \sim \text{Normal} \left(-\frac{1}{2} \sigma_0, \sigma_0 \right). \quad (3.3.14)$$

By following a procedure similar to that used for derivation of the mean and variance of S_n , we get,

$$\begin{aligned}
&\text{cov} \left(\frac{T_1}{\sqrt{n}}, S_n \right) \\
&= \int L_u J(1 - F_u) (A_1(t) - A_2(t)) f_1(t) dt + \int L_c (A_3(t) - A_4(t)) f_3(t) dt \\
&= c_T.
\end{aligned}$$

To complete the proof of this theorem, it is required to show the joint asymptotic normality of T_1/\sqrt{n} and S_n . From (3.2.12) and (3.3.6) we have :

$$\begin{aligned} T_1 &= \sum_{i=1}^n \beta_i, \\ \sqrt{n}S_n &= \sum_{i=1}^n \beta_i^*. \end{aligned}$$

Therefore, for real a_1 and a_2 ,

$$a_1T_1 + a_2\sqrt{n}S_n = \sum_{i=1}^n (a_1\beta_i + a_2\beta_i^*)$$

is a sum of i.i.d. random variables. We have already derived the expectations, variances and covariance of T_1 and $\sqrt{n}S_n$. Since $a_1T_1 + a_2\sqrt{n}S_n$ is a sum of i.i.d. random variables, the asymptotic normality of $a_1T_1 + a_2\sqrt{n}S_n$ follows from Lindeberg-Lévy central limit theorem. For real a_1 and a_2 , the mean and variance are

$$\begin{aligned} E(a_1T_1 + a_2\sqrt{n}S_n) &= a_10 + a_20 \\ &= 0, \end{aligned}$$

and

$$\text{var}(a_1T_1 + a_2\sqrt{n}S_n) = a_1^2n\sigma_T^2 + a_2^2n\sigma_0^2 + 2a_1a_2nc_T. \quad (3.3.15)$$

Now applying the Cramer-Wold device, we have the joint asymptotic normality of T_1/\sqrt{n} and S_n . That is, $(T_1/\sqrt{n}, S_n)$ is asymptotically normal with mean $(0, 0)$ and covariance matrix,

$$\begin{pmatrix} \sigma_0^2 & C_T \\ C_T & \sigma_T^2 \end{pmatrix}.$$

This completes the proof of the Theorem 3.3.1.

As a consequence of Le Cam's third lemma (Hájek and Sidák, 1967), the statistic T/\sqrt{n} converges weakly to a normal distribution with mean C_T and variance σ_T^2 .

Therefore, the efficacy of the PR-class of statistics is given by

$$C_T^2/\sigma_T^2.$$

The next section is devoted to using the efficacies to derive expressions for optimal statistics in the PR and WL-classes.

3.4 The form of Optimal Statistic

This section is devoted to finding statistics in the PR and WL-classes, such that they have the highest efficacies in their classes.

Notice that, like the PR-class of statistics, the WL-class of statistics can also be represented as linear combinations of two statistics :

$$\begin{aligned} T_{WL} &= \int L_{2u} J_u(1 - \hat{S}(t)) d(N_1(t) - N_2(t)) \\ &\quad + \int L_{2c} J_c(1 - \hat{S}(t)) d(N_3(t) - N_4(t)) \\ &= L_{2u}T_{2u} + L_{2c}T_{2c}, \end{aligned} \tag{3.4.1}$$

where the score functions $J_u(\cdot)$ and $J_c(\cdot)$ satisfy the relationship

$$J_u(v) = -\frac{d}{dv} \{(1 - v) J_c(v)\}. \tag{3.4.2}$$

The statistics T_{2u} and T_{2c} corresponding to WL Sign statistic are denoted by T_{su} and T_{sc} respectively. Similarly, statistics T_{2u} and T_{2c} corresponding to WL Wilcoxon signed rank statistic are denoted by T_{wu} and T_{wc} . Also, notice that, T_{1c} given in (3.2.4) is identical to T_{sc} .

In what follows, a statistic with maximum efficacy in a certain class is called an optimal statistic for that class. In case an optimal statistic is a linear combination

of two statistics, the corresponding weights (coefficients) in the linear combination are called optimal weights.

Theorem 3.4.1 is adapted from Theorem 10.2.3 in Serfling (1980). This theorem gives an expression for the efficacy of an arbitrary linear combination of two statistics. The theorem can be used to derive the optimal statistics in the PR and WL-classes.

Theorem 3.4.1 Let T_{1n}^* and T_{2n}^* be two statistics for testing H_0 . Assume that T_{1n}^* and T_{2n}^* satisfy Pitman Conditions given in 10.2.1 in Serfling (1980), and are in standardized forms. Then for $0 \leq \lambda_1, \lambda_2 \leq 1$, statistics of the form,

$$T_{\lambda_1, \lambda_2} = \lambda_1 T_{1n}^* + \lambda_2 T_{2n}^*, \quad 0 \leq \lambda_1, \lambda_2 \leq 1$$

satisfy Pitman conditions. The efficacy of T_{λ_1, λ_2} is given by

$$eff(T_{\lambda_1, \lambda_2}) = \frac{[\frac{\lambda_1}{c_1} + \frac{\lambda_2}{c_2}]^2}{[\lambda_1^2 + \lambda_2^2 + 2\lambda_1\lambda_2\rho]}, \quad (3.4.3)$$

where

$$c_1 = (\text{efficacy of } T_{1n}^*)^{-\frac{1}{2}}, \quad c_2 = (\text{efficacy of } T_{2n}^*)^{-\frac{1}{2}}$$

and

$$\rho = \lim_{n \rightarrow \infty} \text{corr}(T_{1n}^*, T_{2n}^*).$$

Proof. See Serfling (1980).

Notice that if $\lambda_1 + \lambda_2 \neq 0$, then T_{λ_1, λ_2} in Theorem 3.4.1 is equivalent to

$$T_\lambda = (1 - \lambda)T_{1n}^* + \lambda T_{2n}^*, \quad (3.4.4)$$

where,

$$\lambda = \frac{\lambda_2}{\lambda_1 + \lambda_2}.$$

The efficacy of T_λ is given by

$$eff(T_\lambda) = \frac{\left[\frac{(1-\lambda)}{c_1} + \frac{\lambda}{c_2}\right]^2}{[(1-\lambda)^2 + \lambda^2 + 2(1-\lambda)\lambda\rho]},$$

where c_1 , c_2 and ρ are given in Theorem 3.4.1.

The following theorem shows that $\rho = 0$ for statistics in the PR and WL-classes.

Theorem 3.4.2 Let

$$T_{PR} = L_{1u}T_{1u}^* + L_{1c}T_{1c}^*,$$

and

$$T'_{WL} = L_{2u}T_{2u}'^* + L_{2c}T_{2c}'^*,$$

denote, respectively, statistics in the PR and WL-classes. Then under the null hypothesis of bivariate symmetry,

$$\lim_{n \rightarrow \infty} \text{corr}(T_{1u}^*, T_{1c}^*) = 0,$$

and

$$\lim_{n \rightarrow \infty} \text{corr}(T_{2u}'^*, T_{2c}'^*) = 0. \quad (3.4.5)$$

Proof. First we consider the PR-class of statistics. Popovich (1983) proved that T_{1u} and T_{1c} are conditionally independent given $N_u = n_u$, $N_c = n_c$ that is,

$$\text{cov}(T_{1u}^*, T_{1c}^* \mid N_u = n_u, N_c = n_c) = 0. \quad (3.4.6)$$

Again, under H_0 , we have

$$E[T_{1u}^* \mid N_u = n_u, N_c = n_c] = 0,$$

$$E[T_{1c}^* \mid N_u = n_u, N_c = n_c] = 0,$$

so that

$$\text{cov} \left[E(T_{1u}^* \mid N_u = n_u, N_c = n_c), E(T_{1c}^* \mid N_u = n_u, N_c = n_c) \right] = 0. \quad (3.4.7)$$

Therefore, from (3.4.6) and (3.4.7) and the fact that for random variables X , Y and Z ,

$$\text{cov}(X, Y) = E [\text{cov}(X, Y \mid Z)] + \text{cov} [E(X \mid Z), E(Y \mid Z)],$$

it follows that

$$\text{cov}(T_{1u}^*, T_{1c}^*) = 0.$$

Thus, $\rho = 0$ for the PR-class of statistics.

Now we consider the WL-class. Recall from Section 2.2,

$$F(t) = \sum_{j=1}^4 F_j(t).$$

Notice that $T_{2u}^{'*}$ and $T_{2c}^{'*}$, are standardized versions of T_{2u}' and T_{2c}' , respectively, where,

$$\begin{aligned} T_{2u}' &= \int J_u(1 - F(t))d(N_1(t) - N_2(t)) \\ &= \sum_{i=1}^n \epsilon_i [\delta_i J_u(1 - F(|Z_i|))] \\ &= \sum_{i=1}^n \beta_{ui}, \end{aligned} \tag{3.4.8}$$

and

$$\begin{aligned} T_{2c}' &= \int J_c(1 - F(t))d(N_3(t) - N_4(t)) \\ &= \sum_{i=1}^n \epsilon_i [(1 - \delta_i) J_c(1 - F(|Z_i|))] \\ &= \sum_{i=1}^n \beta_{ci}. \end{aligned} \tag{3.4.9}$$

Dabrowska (1990) showed that

$$T_{2u}' - T_{2u} = o_p(1),$$

and

$$T'_{2c} - T_{2c} = o_p(1),$$

where T_{2u} and T_{2c} are given in (3.4.1). Therefore, we can consider T'_{WL} instead of T_{WL} .

As noted in (3.2.13),

$$E[\epsilon_i \mid \delta_i, |Z_i|] = 0,$$

under H_0 . Straightforward calculations similar to those at (3.2.13) gives,

$$E[\beta_{ui} \mid \delta_i, |Z_i|] = 0,$$

$$E[\beta_{ci} \mid \delta_i, |Z_i|] = 0,$$

and

$$E[\beta_{ui} \beta_{cj} \mid \delta_i, \delta_j, |Z_i|, |Z_j|] = 0, \quad 1 \leq i, j, \leq n.$$

Therefore, the conditional covariance of T'_{2u}^* and T'_{2c}^* is

$$\text{cov} [E(T'_{2u}^*, T'_{2c}^* \mid \delta_1, \dots, \delta_n, |Z_1|, \dots, |Z_n|)] = 0.$$

Using the same argument as in the previous case, the correlation coefficient between T'_{2u}^* and T'_{2c}^* for the WL-class can be shown to be 0 under null hypothesis.

The next theorem gives an expression for λ in Theorem 3.4.1 such that the efficacy of T_λ is maximum when the asymptotic correlation between T_{1n} and T_{2n} is zero under the null hypothesis.

Theorem 3.4.3 Assume conditions of Theorem 3.4.1 and suppose that,

$$\lim_{n \rightarrow \infty} \text{corr}(T_{1n}^*, T_{2n}^*) = 0$$

under H_0 . Then statistics of the form T_λ attain maximum efficacy for

$$\lambda = \frac{c_1}{c_1 + c_2}.$$

The maximum efficacy is given by

$$eff(T_\lambda) = \left[\frac{1}{c_1^2} + \frac{1}{c_2^2} \right].$$

Proof. From Theorem 3.4.1, the efficacy of T_λ when $\rho = 0$ is,

$$eff(T_\lambda) = \frac{\left[\frac{(1-\lambda)}{c_1} + \frac{\lambda}{c_2} \right]^2}{[(1-\lambda)^2 + \lambda^2]}.$$

Maximizing $eff(T_\lambda)$ is same as maximizing $\log eff(T_\lambda)$. Now differentiating $\log eff(T_\lambda)$ with respect to λ and equating the resulting expression to 0, we have

$$\frac{c_1 - c_2}{(1-\lambda)c_2 + \lambda c_1} - \frac{1}{2} \frac{4\lambda - 2}{1 + 2\lambda^2 - 2\lambda} = 0. \quad (3.4.10)$$

If $c_1 + c_2 \neq 0$, solving the equation in (3.4.10) we get,

$$\lambda = \frac{c_1}{c_1 + c_2}.$$

Again differentiating the left hand side of (3.4.10) with respect to λ , we have

$$\begin{aligned} & \frac{-(c_1 - c_2)(c_1 - c_2)}{[(1-\lambda)c_2 + \lambda c_1]^2} - \frac{-2(1 + 2\lambda^2 - 2\lambda) - (2\lambda - 1)(4\lambda - 2)}{[1 + 2\lambda^2 - 2\lambda]^2} \\ &= -\frac{(c_1 - c_2)^2}{[(1-\lambda)c_2 + \lambda c_1]^2} - \frac{2[(1 + 2\lambda^2 - 2\lambda) - (2\lambda - 1)^2]}{[1 + 2\lambda^2 - 2\lambda]^2} \\ &= -\frac{(c_1 - c_2)^2}{[(1-\lambda)c_2 + \lambda c_1]^2} - \frac{4\lambda(1-\lambda)}{[1 + 2\lambda^2 - 2\lambda]^2} \\ &< 0, \end{aligned}$$

showing that $eff(T_\lambda)$ is maximized at

$$\lambda = \frac{c_1}{c_1 + c_2}.$$

The maximum efficacy is given by

$$\begin{aligned} eff(T_\lambda) &= \frac{\left[\frac{c_2}{(c_1+c_2)c_1} + \frac{c_1}{(c_1+c_2)c_2} \right]^2}{\left[\left(\frac{c_2}{c_1+c_2} \right)^2 + \left(\frac{c_1}{c_1+c_2} \right)^2 \right]} \\ &= \left[\frac{1}{c_1^2} + \frac{1}{c_2^2} \right], \end{aligned}$$

where c_1 and c_2 are given in Theorem 3.4.1.

Listed below are the variances and efficacies of the various statistics considered thus far. These expressions can be derived from Theorem 3.2.1 and Theorem 3.3.1 and will be needed later in this manuscript. Recall that $F(t)$ appearing in some of the expressions is defined as

$$F(t) = \sum_{j=1}^4 F_j(t).$$

First, to consider the PR-class of statistics, we need the expressions of variances and efficacies of T_{1c} and T_{1u} which are given by

$$\begin{aligned} var(T_{1c}) &= 2 \int f_3(t) dt \\ &= 2 F_3(0), \\ eff(T_{1c}) &= \frac{[\int (A_3(t) - A_4(t)) f_3(t) dt]^2}{2 F_3(0)}, \\ var(T_{1u}) &= 2 \int (1 - F_u(t))^2 f_1(t) dt \\ &= 2 \int \left(1 - \frac{F_1(t)}{F_1(0)} \right)^2 f_1(t) dt \\ &= \frac{2}{3} F_1(0), \\ eff(T_{1u}) &= \frac{[\int (1 - F_u(t)) (A_1(t) - A_2(t)) f_1(t) dt]^2}{\frac{2}{3} F_1(0)}, \end{aligned} \tag{3.4.11}$$

Recall that T_{1c} is identical to T_{sc} . Therefore to consider WL Sign statistic we need the expressions of variance and efficacy of T_{su} in addition to those for T_{sc} .

$$var(T_{su}) = 2 \int f_1(t) dt,$$

$$eff(T_{su}) = \frac{[\int (A_1(t) - A_2(t)) f_1(t) dt]^2}{2 \int (1 - F(t))^2 f_1(t) dt}. \quad (3.4.12)$$

Lastly, for WL Wilcoxon sign statistic, we require the expressions of variance and efficacy of T_{wu} and T_{wc} which are

$$\begin{aligned} var(T_{wu}) &= 2 \int (1 - F(t))^2 f_1(t) dt \\ eff(T_{wu}) &= \frac{[\int (A_1(t) - A_2(t)) (1 - F(t)) f_1(t) dt]^2}{2 \int (1 - F(t))^2 f_1(t) dt}, \end{aligned}$$

and

$$\begin{aligned} var(T_{wc}) &= 2 \int (1 - \frac{1}{2}F(t))^2 f_3(t) dt, \\ eff(T_{wc}) &= \frac{[\int (A_3(t) - A_4(t)) (1 - \frac{1}{2}F(t)) f_3(t) dt]^2}{2 \int (1 - \frac{1}{2}F(t))^2 f_3(t) dt}. \end{aligned} \quad (3.4.13)$$

In the next section, the efficacies of selected members in the PR and WL-classes are compared with the efficacies of optimal statistics in these classes.

3.5 Asymptotic Relative Efficiencies of Various Statistics

As noted in Section 2.3, the model considered by Woolson and Lachenbruch (1980) is

$$\begin{aligned} exp(X'_1) &= aV_1W, \\ exp(X'_2) &= V_2W, \end{aligned} \quad (3.5.1)$$

where $X'_i = \log X_i$, $i = 1, 2$, $a > 0$ is an unknown parameter, V_i $i = 1, 2$, are independent, identically distributed non-negative random variables and W is a non-negative random variable independent of the V_i . From (3.5.1),

$$\begin{aligned} X'_1 &= \log a + \log V_1 + \log W, \\ X'_2 &= \log V_2 + \log W. \end{aligned} \quad (3.5.2)$$

We can rewrite the equations in (3.5.2) in the form :

$$\begin{aligned} X'_1 &= \theta + \eta_1 + \mu, \\ X'_2 &= \eta_2 + \mu, \end{aligned}$$

where η_1, η_2 are independent, identically distributed random variables and μ is a random variable independent of the η_i .

In this section we present the ARE of various statistics under the assumption that the density of η_i is the extreme value density given by

$$f_{\eta_i}(u) = e^u e^{-e^u}, \quad -\infty \leq u \leq \infty, \quad i = 1, 2, \quad (3.5.3)$$

and that of μ is extreme value density given by

$$f_{\mu}(u) = \tau e^{\tau(u-\alpha_1)} e^{-e^{\tau(u-\alpha_1)}}, \quad -\infty \leq u \leq \infty, \quad -\infty < \alpha_1 < \infty, \quad \tau > 0.$$

Assuming extreme value distributions for η_i , $i = 1$ and 2 , we get logistic distribution for $X'_2 - X'_1$. This is very reasonable because the behaviour of random variables with logistic distribution is close to that of random variables with normal distribution.

Furthermore if $C' = \log C$ where C is the censoring time, we assume that the density of C' is also extreme value density given by

$$f_{C'}(u) = \tau e^{\tau(u-\alpha_2)} e^{-e^{\tau(u-\alpha_2)}}, \quad -\infty \leq u \leq \infty, \quad -\infty < \alpha_2 < \infty, \quad \tau > 0.$$

Note that under the above assumptions, the correlation coefficient between X'_1 and X'_2 is given by

$$\begin{aligned}
 \rho &= \frac{\text{cov}(X'_1, X'_2)}{\sqrt{\text{var}(X'_1) \text{var}(X'_2)}} \\
 &= \frac{\frac{\pi^2}{6\tau^2}}{\frac{\pi^2}{6\tau^2} + \frac{\pi^2}{6}} \\
 &= \frac{1}{\tau^2 + 1},
 \end{aligned} \tag{3.5.4}$$

which approaches 1 as $\tau \rightarrow 0$. Under the null hypothesis of symmetry, probability of a doubly censored observation is given by

$$\begin{aligned}
 p_0 &= P[X'_1 > C', X'_2 > C'] \\
 &= P[\eta_1 + \mu > C', \eta_2 + \mu > C'] \\
 &= P[\eta_1 > C' - \mu, \eta_2 > C' - \mu] \\
 &= \int_{-\infty}^{\infty} e^{-2e^z} \frac{\tau e^{\tau(z-\alpha_2+\alpha_1)}}{(1 + e^{\tau(z-\alpha_2+\alpha_1)})} dz.
 \end{aligned}$$

Therefore, the parameters τ , α_1 and α_2 determine the heaviness of censoring. Correlation coefficient ρ depends on τ only. For a fixed value of τ , that is, for a fixed value of ρ , probability of double censoring depends on the values of α_1 and α_2 .

The case of $\rho = 0$ is equivalent to the independent case here,

$$\begin{aligned}
 X'_1 &= \theta + \eta_1, \\
 X'_2 &= \eta_2,
 \end{aligned} \tag{3.5.5}$$

where the distribution of η_i $i = 1, 2$, are give by (3.5.3). Therefore, under H_0 , probability of a doubly censored observation is given by

$$\begin{aligned}
 p_0 &= P[X'_1 > C', X'_2 > C'] \\
 &= \int_{-\infty}^{\infty} \tau e^{\tau(c-\alpha)-e^{\tau(c-\alpha)}-2e^c} dc.
 \end{aligned}$$

For the purpose of efficacy calculation, the parameter τ is so chosen that the correlation coefficient between the underlying log-failure times is $\rho = 0, 0.10, 0.25, 0.50$ and 0.75 . Further, for each of these values of ρ , the parameters α_1 and α_2 are selected in such way that under the null hypothesis, the probability of a doubly censored pair is equal to $p_0 = 0.10, 0.25, 0.50$ and 0.75 . The following statistics are considered for numerical comparison of their efficacies.

1. PR statistic with equal weights (PR-EQ).
2. PR statistic with weights proportional to the standard deviations (PR-STD).
3. PR statistic with optimal weights (PR-OPT).
4. WL Sign statistic with equal weights (S-EQ).
5. WL Sign statistic with weights proportional to the standard deviations (S-STD).
6. WL Sign statistic with optimum weights (S-OPT).
7. WL Wilcoxon signed rank statistic with equal weights (W-EQ).
8. WL Wilcoxon signed rank statistic with weights proportional to the standard deviations (W-STD).
9. WL Wilcoxon signed rank statistic with optimal weights (W-OPT).

Last six statistics in the list are from the WL-class.

Table 3.1–Table 3.4, given on pages 51–54 contain the asymptotic relative efficiencies (ARE) of the above nine statistics. Tables 3.1–3.4 can be used to calculate the ARE of any two statistics given in the list.

The figures, Fig 3.1–Fig 3.5 on pages 55–59 show the efficacies of the nine statistics when $\rho = 0, 0.10, 0.25, 0.50$ and 0.75 .

From Table 3.1, in the independent case ($\rho = 0$), and when the probability of double censoring is low, S-EQ is almost as efficient as S-OPT. The loss in efficacy is only 2.68% when $p_0 = 0.10$ and $\rho = 0$. But, S-STD is performing better as the probability of double censoring increases. For a fixed value of p_0 and non-zero correlation coefficient ρ , performance of S-EQ improves with increasing value of ρ . But, for a fixed value of p_0 , performance of S-STD goes down with increase in ρ . If optimal weights cannot be used, then for lower values of p_0 , S-EQ should be used. For higher values of p_0 , S-STD is preferable.

In Table 3.2, in the independent case, W-EQ has high efficiency when compared to W-OPT for low value of p_0 . For $p_0 = 0.10$ and $\rho = 0$, the loss of efficacy resulting from using W-EQ instead of W-OPT is only 1.25%. As p_0 increases, loss in efficacy resulting from using W-STD instead of the optimal statistic in this class, W-OPT, is less than that resulting from using W-EQ instead of W-OPT. When p_0 is low, either W-EQ or W-STD could be used instead of W-OPT. For $p_0 = 0.10$, efficacy loss by using W-EQ is less than 8%, whereas the loss never exceeds 15% if W-STD is used instead of W-OPT. In general, for low values of p_0 and high values of ρ , W-EQ is preferable over W-STD. But for high values of p_0 W-STD is performing better irrespective of the magnitude of correlation coefficient.

Comparing statistics belonging to the PR-class from Table 3.3, almost always PR-STD is performing better than PR-EQ. The only exceptions are when $p_0 = 0.10$ and correlation coefficient is either very low or very high. In these cases, PR-EQ is almost as efficient as PR-OPT. Particularly for $p_0 = 0.10$, by using PR-EQ instead of PR-OPT, the loss in efficacy is less than 7.26%. In general, the use of PR-STD

is recommended. The loss in efficacy resulting from the use of PR-STD instead of PR-OPT is never more than 22%.

From the Table 3.4, one can see that when $\rho = 0$, W-OPT is more efficient than the other optimal statistics. All three statistics compared in this table have efficacies within 1% of each other for high value of p_0 in the independent case. In general, efficacy of PR-OPT is slightly higher than the efficacy of optimal statistics in the WL-class.

By examining the efficacies of the nine statistics given in Tables 3.1–3.4 and displayed in Figures 3.1–3.5, it is evident that the members of the PR-class are performing very well in almost every case. Also, for small sample sizes, exact test can be performed for members of this class of statistics. When construction of optimal statistics is not possible, depending on heaviness of censoring and degree of association in the pairs, decision has to be made about using PR statistic with equal weights or with weights proportional to standard deviations. In general, when the probability of double censoring is low, PR-EQ is recommended but for higher percentage of doubly censored pairs in the sample, PR-STD is preferable.

Table 3.1. ARE of S-EQ and S-STD with respect to S-OPT (denoted by ARE (i) and ARE (ii)).

p_0	ARE	ρ				
		0	0.10	0.25	0.50	0.75
0.10	(i)	0.97313	0.88831	0.95639	0.98947	0.99181
	(ii)	0.81839	0.78922	0.81070	0.79269	0.71277
0.25	(i)	0.71128	0.63964	0.72288	0.80301	0.84046
	(ii)	0.76117	0.70965	0.68951	0.64460	0.55690
0.50	(i)	0.62495	0.53139	0.56235	0.61371	0.65178
	(ii)	0.82248	0.79317	0.73470	0.63457	0.50993
0.75	(i)	0.53421	0.50480	0.51459	0.53496	0.55072
	(ii)	0.90504	0.89858	0.83849	0.71376	0.55388

Table 3.2. ARE of W-EQ and W-STD with respect to optimal W-OPT (denoted by ARE (i) and ARE (ii)).

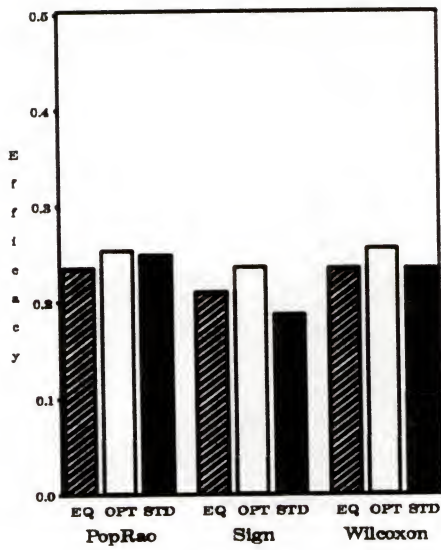
p_0	ARE	ρ				
		0	0.10	0.25	0.50	0.75
0.10	(i)	0.98753	0.91985	0.97963	0.99937	0.99995
	(ii)	0.92778	0.91777	0.93173	0.91353	0.84253
0.25	(i)	0.73659	0.66074	0.75327	0.83904	0.87781
	(ii)	0.86574	0.81672	0.80806	0.77266	0.68672
0.50	(i)	0.63777	0.53536	0.56965	0.62618	0.66746
	(ii)	0.87550	0.84018	0.79093	0.70412	0.58363
0.75	(i)	0.53630	0.50513	0.51550	0.53694	0.55313
	(ii)	0.91808	0.91065	0.85567	0.73895	0.58046

Table 3.3. ARE of PR-EQ and PR-STD with respect to PR-OPT (denoted by ARE (i) and ARE (ii)).

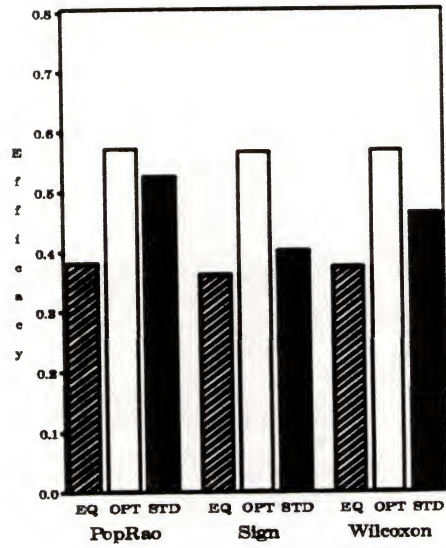
p_0	ARE	ρ				
		0	0.10	0.25	0.50	0.75
0.10	(i)	0.99186	0.92736	0.98152	0.99919	0.99968
	(ii)	0.98873	0.98111	0.98768	0.97420	0.91475
0.25	(i)	0.74935	0.66725	0.76041	0.84265	0.87839
	(ii)	0.95456	0.91792	0.92255	0.90312	0.83178
0.50	(i)	0.65135	0.53889	0.57577	0.63488	0.67670
	(ii)	0.96269	0.92936	0.91110	0.87019	0.78852
0.75	(i)	0.54435	0.50647	0.51836	0.54223	0.56007
	(ii)	0.97407	0.96503	0.94383	0.89282	0.80241

Table 3.4. ARE of PR-OPT with respect to W-OPT and S-OPT (denoted by ARE (i) and ARE (ii)).

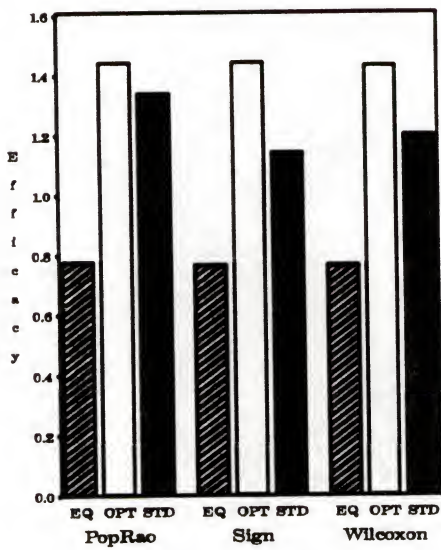
p_0	ARE	ρ				
		0	0.10	0.25	0.50	0.75
0.10	(i)	0.98112	0.99321	1.01768	1.03522	1.04729
	(ii)	1.12185	1.07298	1.10958	1.13946	1.13924
0.25	(i)	0.97441	1.00542	1.02191	1.04029	1.05422
	(ii)	1.02119	1.00917	1.02238	1.03888	1.04759
0.50	(i)	0.98607	1.00782	1.01435	1.02457	1.03339
	(ii)	1.00777	1.00053	1.00188	1.00554	1.00896
0.75	(i)	0.99598	1.00302	1.00539	1.00927	1.01148
	(ii)	1.00080	1.00002	1.00012	1.00056	1.00105



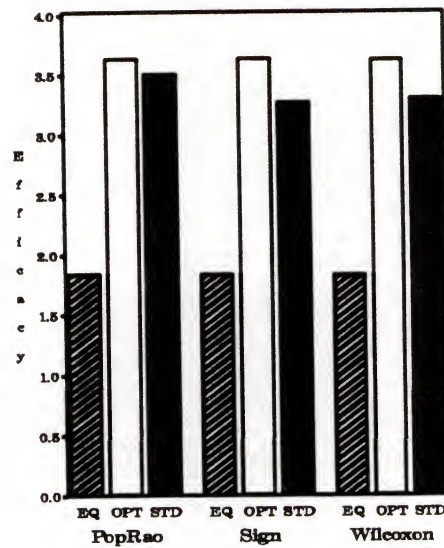
(a)



(b)

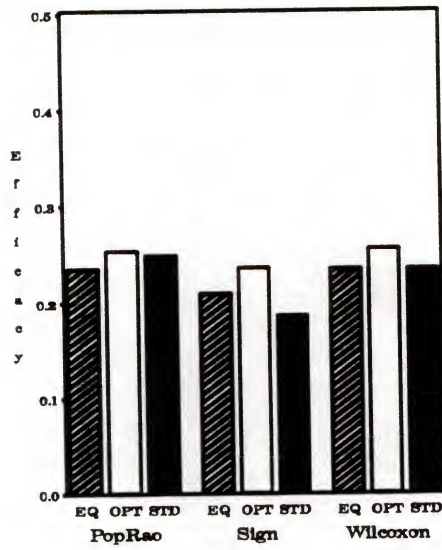


(c)

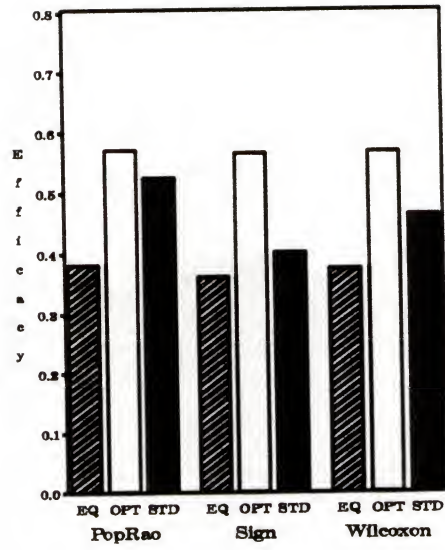


(d)

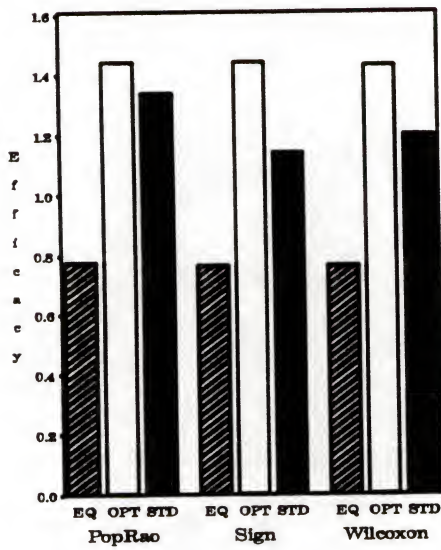
Fig 3.1. Efficacy Comparison for $\rho = 0$
 (a) $p_0 = 0.10$; (b) $p_0 = 0.25$;
 (c) $p_0 = 0.50$; (d) $p_0 = 0.75$;



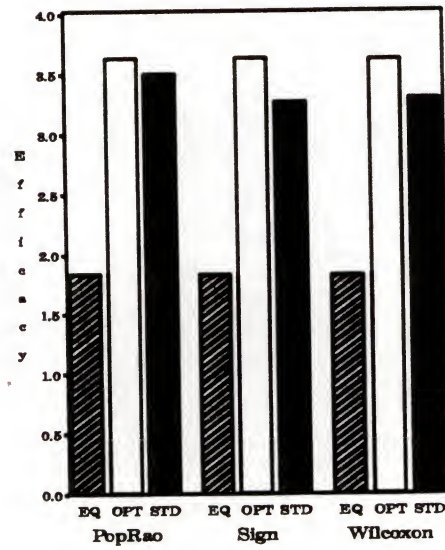
(a)



(b)

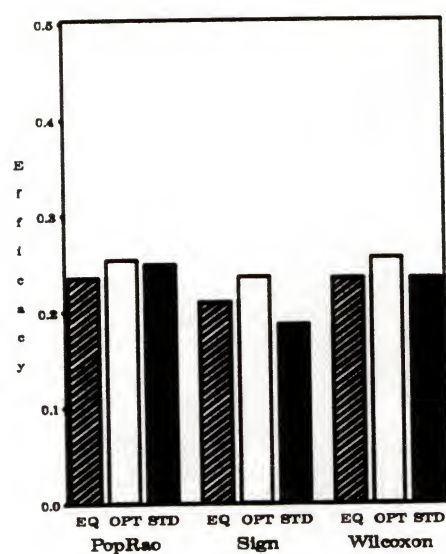


(c)

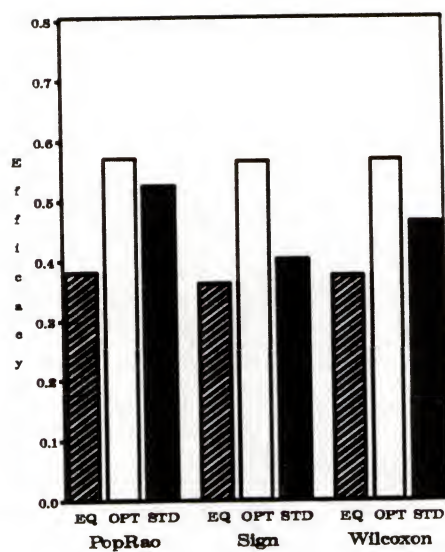


(d)

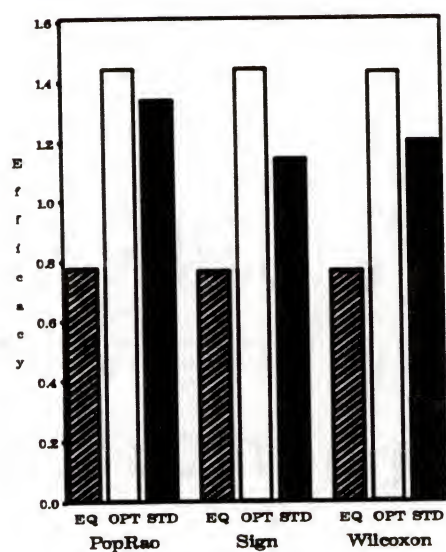
Fig 3.2. Efficacy Comparison for $\rho = 0.10$
 (a) $p_0 = 0.10$; (b) $p_0 = 0.25$;
 (c) $p_0 = 0.50$; (d) $p_0 = 0.75$;



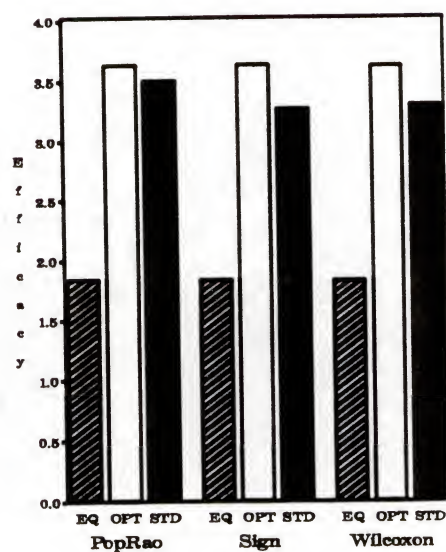
(a)



(b)

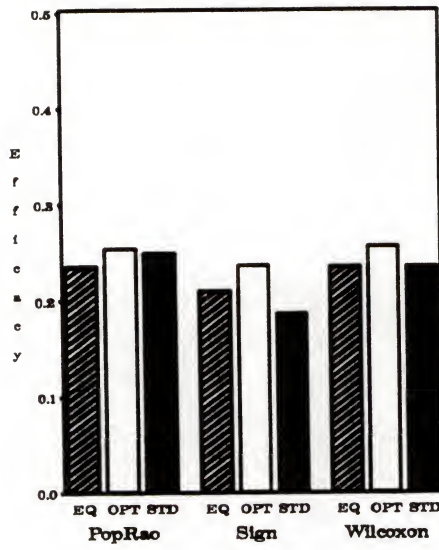


(c)

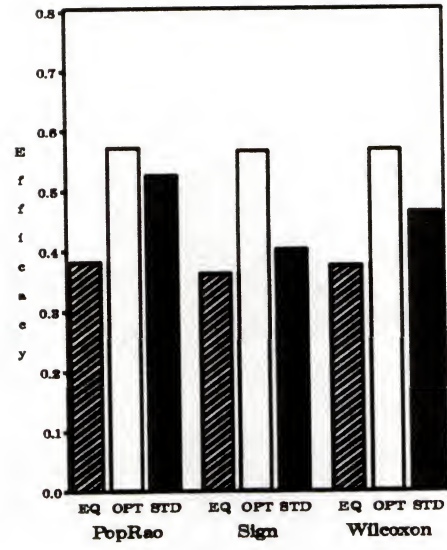


(d)

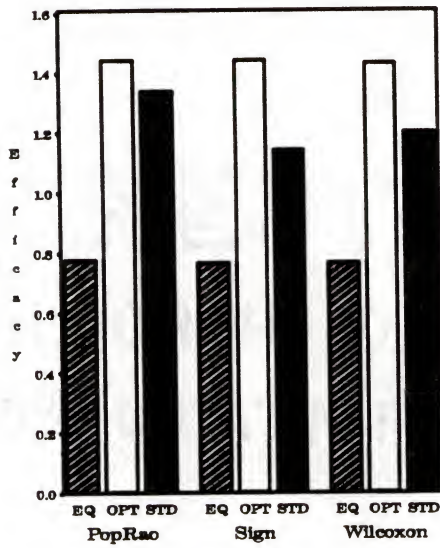
Fig 3.3. Efficacy Comparison for $\rho = 0.25$
 (a) $p_0 = 0.10$; (b) $p_0 = 0.25$;
 (c) $p_0 = 0.50$; (d) $p_0 = 0.75$;



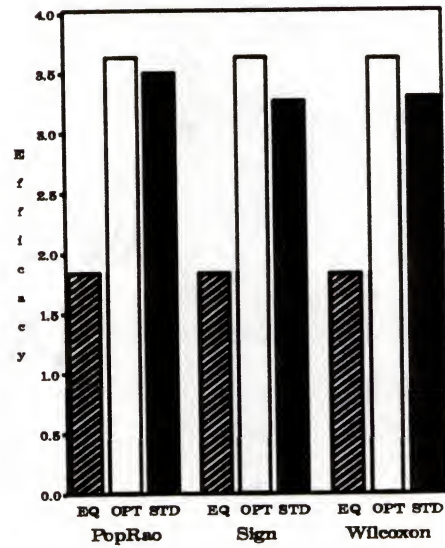
(a)



(b)

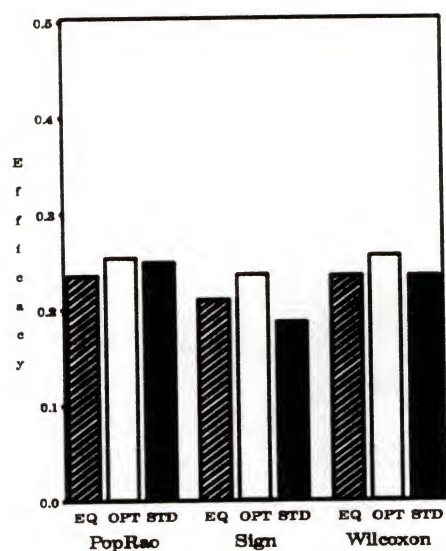


(c)

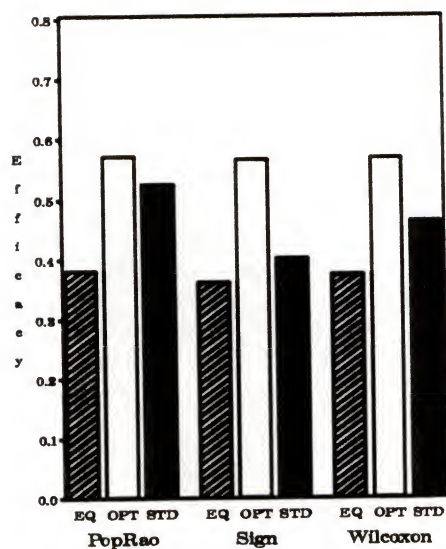


(d)

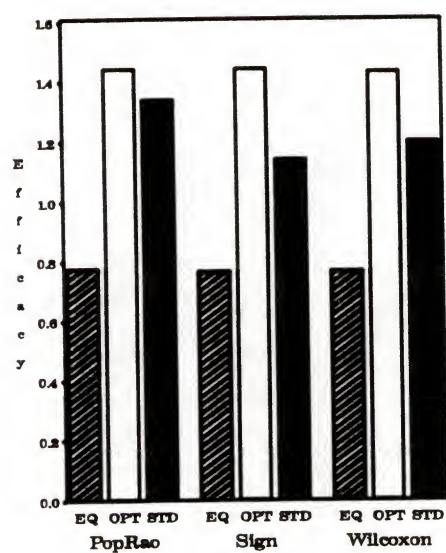
Fig 3.4. Efficacy Comparison for $\rho = 0.50$
 (a) $p_0 = 0.10$; (b) $p_0 = 0.25$;
 (c) $p_0 = 0.50$; (d) $p_0 = 0.75$;



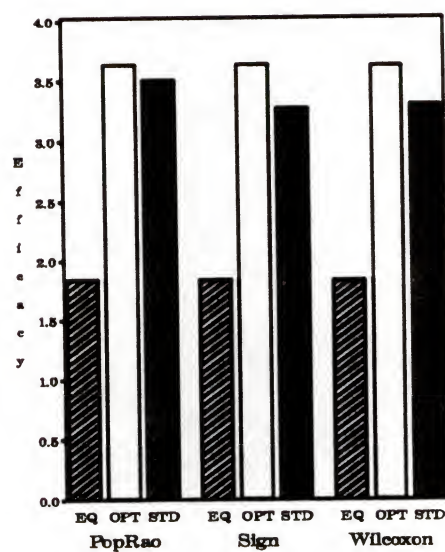
(a)



(b)



(c)



(d)

Fig 3.5. Efficacy Comparison for $\rho = 0.75$
 (a) $p_0 = 0.10$; (b) $p_0 = 0.25$;
 (c) $p_0 = 0.50$; (d) $p_0 = 0.75$;

CHAPTER 4

ESTIMATION OF OPTIMAL WEIGHTS

4.1 Introduction

In Chapter 3, we presented a comparison of the efficacies of statistics in the PR and WL-classes. We noted that, depending upon the heaviness of double censoring and the correlation between the survival times, optimal statistics can have very high efficacy compared to other members in a class.

Since the weights needed to compute the optimal statistics depend on the unknown joint distribution of the observed survival times in a complicated manner, practical use of these statistics requires reasonable estimates of the optimal weights. In this chapter we describe a method of performing tests based on optimal statistics with estimated weights. In Section 4.2, a model is proposed for the conditional distributions of the $|Z_i|$. Section 4.3 has theorems needed to justify the application of the proposed model to estimate the optimal weights while Section 4.4 has some of the estimates of optimal weights for selected distributional forms. Lastly, Section 4.5 has the forms of statistics with estimated optimal weights.

4.2 Modelling Conditional Distributions

It is seen from Theorem 3.4.3 that the weights associated with optimal statistics depend upon the joint distribution of (Y_1, Y_2) in a complicated way. Specifying

realistic joint distributions for (Y_1, Y_2) is difficult because such a specification involves the joint distribution of (X_1, X_2) and C . In this chapter, we will construct estimates of optimal weights assuming the following model for the conditional distributions of $|Z_i|$ given $i \in B_j$, $j = 1, 2, 3, 4$.

Consider the following model for the conditional distribution of $|Z_i|$ given $i \in B_j$, $j = 1, 2, 3, 4$.

$$\begin{aligned} P(|Z_i| \geq t \mid i \in B_1) &= 2[1 - H(t)], \\ P(|Z_i| \geq t \mid i \in B_2) &= 2[1 - H(t\theta)], \\ P(|Z_i| \geq t \mid i \in B_3) &= 2[1 - H(t\nu)], \\ P(|Z_i| \geq t \mid i \in B_4) &= 2[1 - H(t\nu\theta)], \end{aligned} \quad (4.2.1)$$

where $H(\cdot)$ is a c.d.f. of a random variable symmetric about zero.

The model in (4.2.1) is a reasonable model from a practical point of view for two reasons. First of all, the observed data $\{|Z_i| \mid i \in B_j\}$, for $j = 1, 2, 3, 4$, can be used to check the adequacy of the model for various choices of H . A test of fit or a graphical approach such as q-q plot can be used for this purpose. Secondly, the model not only possesses a simple structure, but also is flexible enough to represent a wide variety of distributional forms. Some important characteristics of the model are listed below.

Under H_0 ,

$$\{|Z_i| \mid i \in B_1\} \stackrel{d}{=} \{|Z_i| \mid i \in B_2\}$$

and

$$\{|Z_i| \mid i \in B_3\} \stackrel{d}{=} \{|Z_i| \mid i \in B_4\},$$

so that $\theta = 1$ corresponds to the null hypothesis. Note that $\{|Z_i| \mid i \in B_1\}$ and $\{|Z_i| \mid i \in B_3\}$ need not be the same even under H_0 . The same is true for $\{|Z_i| \mid i \in B_2\}$ and $\{|Z_i| \mid i \in B_4\}$. Thus, the model accounts for the fact that under random

censoring, the distribution of $|Z_i|$ from uncensored pairs can be different from the distribution of $|Z_i|$ from singly censored pairs.

If X is random variable with a density $f(\cdot)$, symmetric about 0 on $(-\infty, \infty)$, then the density of $|X|$ is $2f(\cdot)$ on $[0, \infty)$. In what follows, we shall refer to $2f(\cdot)$ as the folded distribution of X . The distributions in (4.2.1) are the folded distributions of random variables with survival distribution $2[1 - H(t)]$.

Fig 4.1 and Fig 4.2 in pages 82–83 represent the density functions corresponding to standard normal distribution and a folded standard normal distribution.

From the model in (4.2.1), the subsurvival distributions of $\{|Z_i|, i \in B_j\}$, $j = 1, 2, 3$ and 4 can be obtained as

$$\begin{aligned} F_{1\theta}(t) &= 2F_{1\theta}(0) [1 - H(t)], \\ F_{2\theta}(t) &= 2F_{2\theta}(0) [1 - H(t\theta)], \\ F_{3\theta}(t) &= 2F_{3\theta}(0) [1 - H(t\nu)], \\ F_{4\theta}(t) &= 2F_{4\theta}(0) [1 - H(t\nu\theta)]. \end{aligned} \tag{4.2.2}$$

Notice that under H_0 the subsurvival functions are denoted by $F_j(t)$. The improper densities corresponding to (4.2.2) are given by

$$\begin{aligned} f_{1\theta}(t) &= 2F_{1\theta}(0) h(t), \\ f_{2\theta}(t) &= 2F_{2\theta}(0) \theta h(t\theta), \\ f_{3\theta}(t) &= 2F_{3\theta}(0) \nu h(t\nu), \\ f_{4\theta}(t) &= 2F_{4\theta}(0) \nu\theta h(t\nu\theta). \end{aligned} \tag{4.2.3}$$

The conditional distribution of Z_i given that it is from an uncensored pair is as follows,

$$\begin{aligned}
P(Z_i \geq t \mid i \in B_1 \cup B_2) &= \begin{cases} \frac{P(Z_i \geq t, i \in B_1)}{P(i \in B_1 \cup B_2)} & \text{if } t > 0 \\ 1 - \frac{P(Z_i \geq t, i \in B_2)}{P(i \in B_1 \cup B_2)} & \text{if } t < 0 \end{cases} \\
&= \begin{cases} \frac{P(i \in B_1)}{P(i \in B_1) + P(i \in B_2)} P(|Z_i| \geq t \mid i \in B_1) & \text{if } t > 0 \\ \frac{P(i \in B_2)}{P(i \in B_1) + P(i \in B_2)} P(|Z_i| \geq -t \mid i \in B_2) & \text{if } t < 0 \end{cases} \\
&= \begin{cases} \frac{P(i \in B_1)}{P(i \in B_1) + P(i \in B_2)} 2[1 - H(t)] & \text{if } t > 0 \\ 1 - \frac{P(i \in B_2)}{P(i \in B_1) + P(i \in B_2)} 2[1 - H(-t\theta)] & \text{if } t < 0. \end{cases}
\end{aligned}$$

Thus, the conditional density of Z , given the difference is uncensored is given by

$$\begin{aligned}
f(t \mid i \in B_1 \cup B_2) &= \begin{cases} \frac{P(i \in B_1)}{P(i \in B_1) + P(i \in B_2)} 2h(t) & \text{if } t > 0 \\ \frac{P(i \in B_2)}{P(i \in B_1) + P(i \in B_2)} \theta 2h(-t\theta) & \text{if } t < 0 \end{cases} \\
&= \begin{cases} \frac{F_{1\theta}(0)}{F_{1\theta}(0) + F_{2\theta}(0)} 2h(t) & \text{if } t > 0 \\ \frac{F_{2\theta}(0)}{F_{1\theta}(0) + F_{2\theta}(0)} \theta 2h(-t\theta) & \text{if } t < 0. \end{cases} \tag{4.2.4}
\end{aligned}$$

When $H_0 : \theta = 1$ is true, (4.2.4) represents a symmetric distribution. Fig 4.3 on page 84 shows the density of an uncensored Z_i when $h(\cdot)$ is the standard normal density and $\theta = 1.1$. Notice that there is a point of discontinuity at $Z_i = 0$, but c.d.f.s of $\{|Z_i|, i \in B_j\}$, $j = 1, 2, 3$ and 4 are continuous.

The conditional distribution of the censored differences can be obtained in a similar way. The density function is given by

$$\begin{aligned}
f(t \mid i \in B_3 \cup B_4) &= \begin{cases} \frac{P(i \in B_3)}{P(i \in B_3) + P(i \in B_4)} \nu 2h(t\nu) & \text{if } t > 0 \\ \frac{P(i \in B_4)}{P(i \in B_3) + P(i \in B_4)} \nu \theta 2h(-t \nu \theta) & \text{if } t < 0 \end{cases} \\
&= \begin{cases} \frac{F_{3\theta}(0)}{F_{3\theta}(0) + F_{4\theta}(0)} \nu 2h(t\nu) & \text{if } t > 0 \\ \frac{F_4(0)}{F_{3\theta}(0) + F_{4\theta}(0)} \nu \theta 2h(-t\nu\theta) & \text{if } t < 0. \end{cases} \quad (4.2.5)
\end{aligned}$$

Recall from Theorem 3.4.1, that optimal weights depend on the following five integrals,

$$\begin{aligned}
I_1 &= \int_0^\infty (A_1(t) - A_2(t)) f_1(t) dt, \\
I_2 &= \int_0^\infty (A_3(t) - A_4(t)) f_3(t) dt, \\
I_3 &= \int_0^\infty (A_1(t) - A_2(t)) \left(1 - \frac{F_1(t)}{F_1(0)}\right) f_1(t) dt, \\
I_4 &= \int_0^\infty (A_1(t) - A_2(t)) (1 - F(t)) f_1(t) dt, \\
I_5 &= \int_0^\infty (A_3(t) - A_4(t)) \left(1 - \frac{1}{2}F(t)\right) f_3(t) dt. \quad (4.2.6)
\end{aligned}$$

where $A_j(t)$ are defined in (3.3.2) and

$$F(t) = \sum_{j=1}^4 P(|Z_i| \geq t, i \in B_j). \quad (4.2.7)$$

Using (4.2.3) in the definitions of $A_j(t)$, we get,

$$\begin{aligned}
A_1(t) - A_2(t) &= \frac{d}{d\theta} [\log f_{1\theta}(t) - \log f_{2\theta}(t)] \Big|_{\theta=1} \\
&= \frac{d}{d\theta} \left[\log \frac{F_{1\theta}(0)}{F_{2\theta}(0)} - \log \theta h(t\theta) \right] \Big|_{\theta=1},
\end{aligned}$$

and

$$\begin{aligned}
A_3(t) - A_4(t) &= \frac{d}{d\theta} [\log f_{3\theta}(t) - \log f_{4\theta}(t)] \Big|_{\theta=1} \\
&= \frac{d}{d\theta} \left[\log \frac{F_{3\theta}(0)}{F_{3\theta}(0)} - \log \nu \theta h(t\nu\theta) \right] \Big|_{\theta=1}. \quad (4.2.8)
\end{aligned}$$

Thus, in order to evaluate the integrals in (4.2.6), in addition to the form of $h(\cdot)$, we need to specify the derivatives of

$$\log \frac{F_{1\theta}(0)}{F_{2\theta}(0)} \quad \text{and} \quad \log \frac{F_{3\theta}(0)}{F_{4\theta}(0)}$$

at $\theta = 1$.

Now,

$$\frac{F_{1\theta}(0)}{F_{2\theta}(0)} = \left[\frac{F_{1\theta}(0)}{F_{1\theta}(0) + F_{2\theta}(0)} \bigg/ \frac{F_{2\theta}(0)}{F_{1\theta}(0) + F_{2\theta}(0)} \right], \quad (4.2.9)$$

$$\frac{F_{3\theta}(0)}{F_{4\theta}(0)} = \left[\frac{F_{3\theta}(0)}{F_{3\theta}(0) + F_{4\theta}(0)} \bigg/ \frac{F_{4\theta}(0)}{F_{3\theta}(0) + F_{4\theta}(0)} \right]. \quad (4.2.10)$$

Therefore, (4.2.9) represents the odds ratio of observing a positive difference with respect to a negative difference given that the difference corresponds to an uncensored pair. Similarly, (4.2.10) represents the odds ratio of observing a positive difference with respect to a negative difference given that the difference comes from singly censored pair. We will refer to (4.2.9) and (4.2.10) as the odds ratio for uncensored and censored differences respectively.

Let $g(\cdot)$ be a function such that $g(\theta)$ is differentiable at $\theta = 1$ and $g(1) = 0$. We assume that the odds ratios at (4.2.9) and (4.2.10) have the forms,

$$\frac{F_{1\theta}(0)}{F_{2\theta}(0)} = \exp(g(\theta)),$$

$$\frac{F_{3\theta}(0)}{F_{4\theta}(0)} = \exp(\nu g(\theta)). \quad (4.2.11)$$

Note that, the forms for the odds ratios specified in (4.2.11) imply that $g'(1)$ and $\nu g'(1)$ are the rates at which these ratios are changing in the neighborhood of $H_0 : \theta = 1$. Also, the parameter ν used in (4.2.11) is same as the one used in (4.2.1).

It follows that the optimal weights under (4.2.1) and (4.2.11) depend upon, $h(\cdot)$, $g'(1)$ and ν . Since $2h(\cdot)$ represents the conditional density of $|Z|$ under H_0 , any density on $[0, \infty]$ is a possible candidate for $2h(\cdot)$. For example, the exponential, folded standard normal, folded logistic etc. are suitable candidates. The value of ν can be consistently estimated from the data, by noting that

$$\nu = \log \frac{F_{3\theta}(0)}{F_{4\theta}(0)} / \log \frac{F_{1\theta}(0)}{F_{2\theta}(0)}. \quad (4.2.12)$$

Since $F_{j\theta}(0)$, $j = 1, 2, 3, 4$, can be consistently estimated by

$$\begin{aligned} \hat{F}_{1\theta}(0) &= N_{u+} / n, \\ \hat{F}_{2\theta}(0) &= N_{u-} / n, \\ \hat{F}_{3\theta}(0) &= N_{c+} / n, \\ \hat{F}_{4\theta}(0) &= N_{c-} / n, \end{aligned} \quad (4.2.13)$$

a consistent estimate of ν is given by

$$\hat{\nu} = \frac{\log N_{c+} - \log N_{c-}}{\log N_{u+} - \log N_{u-}}. \quad (4.2.14)$$

The value of $g'(1)$ needs to be specified by the user based on the desired rate of departure of the alternatives from the null hypothesis. Higher values of $g'(1)$ correspond to more severe departures. Let $g'(1) = \alpha$ and we will refer to α as the rate of departure from the null hypothesis. Then from (4.2.11),

$$\begin{aligned} \frac{d}{d\theta} \left[\log \frac{F_{1\theta}(0)}{F_{2\theta}(0)} \right] \Big|_{\theta=1} &= g'(1) = \alpha, \\ \frac{d}{d\theta} \left[\log \frac{F_{3\theta}(0)}{F_{4\theta}(0)} \right] \Big|_{\theta=1} &= \nu g'(1) = \nu \alpha. \end{aligned}$$

4.3 Evaluation of the Integrals

The theorems in this section gives expressions for $I_1 - I_5$ in (4.2.6) under the assumption that (4.2.2) and (4.2.11) hold. The expressions are given in terms of a parametric folded density function $h(\cdot)$, the rate of departure α and censoring effect constant ν . In Section 4.4, actual forms of $I_1 - I_5$ will be given when $h(\cdot)$ is an exponential, a folded standard normal or a folded logistic density function.

Theorem 4.3.1 Let $\alpha = g'(1)$ and,

$$\beta = -2 \int \frac{d}{d\theta} [\log \theta h(t\theta)] \Big|_{\theta=1} h(t) dt.$$

Under (4.2.2) and (4.2.11),

$$I_1 = \int_0^\infty (A_1(t) - A_2(t)) f_1(t) dt = \alpha F_1(0) + \beta F_1(0),$$

$$I_2 = \int_0^\infty (A_3(t) - A_4(t)) f_3(t) dt = \alpha \nu F_3(0) + \beta F_3(0).$$

Proof. By definition,

$$\begin{aligned} A_1(t) - A_2(t) &= \frac{d}{d\theta} [\log f_{1\theta}(t) - \log f_{2\theta}(t)] \Big|_{\theta=1} \\ &= \frac{d}{d\theta} \left[\log \frac{F_{1\theta}(0)}{F_{2\theta}(0)} - \log \theta h(t\theta) \right] \Big|_{\theta=1} \\ &= \alpha - \frac{d}{d\theta} [\log \theta h(t\theta)] \Big|_{\theta=1}. \end{aligned}$$

Therefore,

$$\begin{aligned} I_1 &= \int (A_1(t) - A_2(t)) f_1(t) dt \\ &= \alpha F_1(0) - 2F_1(0) \int \frac{d}{d\theta} [\log \theta h(t\theta)] \Big|_{\theta=1} h(t) dt \\ &= \alpha F_1(0) + \beta F_1(0). \end{aligned}$$

Similarly, we have

$$\begin{aligned} I_2 &= \int (A_3(t) - A_4(t)) f_3(t) dt \\ &= \alpha \nu F_3(0) - 2F_3(0) \int \frac{d}{d\theta} [\log \nu \theta h(t\nu\theta)] \Big|_{\theta=1} \nu h(t\nu) dt. \end{aligned}$$

Notice that

$$\begin{aligned} &\frac{d}{d\theta} [\log \nu \theta h(t\nu\theta)] \Big|_{\theta=1} \\ &= \frac{d}{d\theta} [\log \nu] \Big|_{\theta=1} + \frac{d}{d\theta} [\log \theta h(t\nu\theta)] \Big|_{\theta=1} \\ &= \frac{d}{d\theta} [\log \theta h(t\nu\theta)] \Big|_{\theta=1}. \end{aligned} \tag{4.3.1}$$

Using the result in (4.3.1) and substituting, $t' = t\nu$ in the second term of I_2 , we get

$$\begin{aligned} &2F_3(0) \int \frac{d}{d\theta} [\log \nu \theta h(t\nu\theta)] \Big|_{\theta=1} \nu h(t\nu) dt \\ &= 2F_3(0) \int \frac{d}{d\theta} [\log \theta h(t'\theta)] \Big|_{\theta=1} h(t') dt' \\ &= 2F_3(0) \beta. \end{aligned}$$

Therefore,

$$I_2 = \alpha \nu F_3(0) + \beta F_3(0).$$

The next theorem gives an expression for I_3 in (4.2.6).

Theorem 4.3.2 Let α and β be as in the Theorem 4.3.1 and γ be defined by

$$\gamma = 4 \int_0^\infty \frac{d}{d\theta} [\log \theta h(t\theta)] \Big|_{\theta=1} [1 - H(t)] h(t) dt.$$

Under (4.2.2) and (4.2.11),

$$\begin{aligned} I_3 &= \int_0^\infty (A_1(t) - A_2(t)) \left(1 - \frac{F_1(t)}{F_1(0)}\right) f_1(t) dt \\ &= \frac{\alpha}{2} F_1(0) + (\beta + \gamma) F_1(0). \end{aligned}$$

Proof. From Theorem 4.3.1, (4.2.2), (4.2.3) and (4.2.8), we can write,

$$\begin{aligned} I_3 &= \int (A_1(t) - A_2(t)) f_1(t) dt - \int (A_1(t) - A_2(t)) \frac{F_1(t)}{F_1(0)} f_1(t) dt \\ &= \alpha F_1(0) + \beta F_1(0) - 4F_1(0) \int_0^\infty \left(\alpha - \frac{d}{d\theta} [\log \theta h(t\theta)] \Big|_{\theta=1} \right) [1 - H(t)] h(t) dt. \end{aligned}$$

Using the transformation $[1 - H(t)] = x$, we get

$$\begin{aligned} I_3 &= \alpha F_1(0) + \beta F_1(0) - 4F_1(0) \int_0^{1/2} \alpha x dx \\ &\quad + 4F_1(0) \int_0^\infty \frac{d}{d\theta} [\log \theta h(t\theta)] \Big|_{\theta=1} [1 - H(t)] h(t) dt \\ &= \alpha F_1(0) + \beta F_1(0) - \frac{1}{2} \alpha F_1(0) + 4F_1(0) \int_0^\infty \frac{d}{d\theta} [\log \theta h(t\theta)] \Big|_{\theta=1} [1 - H(t)] h(t) dt \\ &= \frac{\alpha}{2} F_1(0) + (\beta + \gamma) F_1(0). \end{aligned}$$

The next theorem gives expressions for I_4 and I_5 in (4.2.6).

Theorem 4.3.3 Let α, β and γ be as in Theorems 4.3.1 and 4.3.2. Under (4.2.2) and (4.2.11),

$$\begin{aligned} I_4 &= \int_0^\infty (A_1(t) - A_2(t)) (1 - F(t)) f_1(t) dt \\ &= (\alpha + \beta) F_1(0) - (\alpha - 2\gamma) F_1^2(0) \\ &\quad - 8F_1(0)F_3(0) \int_0^\infty \left(\alpha - \frac{d}{d\theta} [\log \theta h(t\theta)] \Big|_{\theta=1} \right) [1 - H(t\theta)] h(t) dt, \end{aligned}$$

and

$$I_5 = \int_0^\infty (A_3(t) - A_4(t)) \left(1 - \frac{1}{2} F(t) \right) f_3(t) dt$$

$$\begin{aligned}
&= (\alpha\nu + \beta) F_3(0) \\
&\quad - 4F_1(0)F_3(0) \int_0^\infty \left(\alpha\nu - \frac{d}{d\theta} [\log \nu \theta h(t\nu\theta)] \Big|_{\theta=1} \right) [1 - H(t\nu)] h(t\nu) dt \\
&\quad - 4F_3^2(0) \int_0^\infty \left(\alpha\nu - \frac{d}{d\theta} [\log \nu \theta h(t\nu\theta)] \Big|_{\theta=1} \right) [1 - H(t\nu)] h(t\nu) dt.
\end{aligned}$$

Proof. Recall from (4.2.7), that under H_0 ,

$$F(t) = 2 (F_1(t) + F_3(t)).$$

Therefore,

$$\begin{aligned}
I_4 &= \int_0^\infty (A_1(t) - A_2(t)) (1 - F(t)) f_1(t) dt \\
&= \int (A_1(t) - A_2(t)) f_1(t) dt - 2 F_1(0) \int (A_1(t) - A_2(t)) F_1(t) f_1(t) dt \\
&\quad - 2 F_3(0) \int (A_1(t) - A_2(t)) F_3(t) f_1(t) dt.
\end{aligned}$$

Using Theorems 4.3.1 and 4.3.2,

$$\begin{aligned}
I_4 &= (\alpha + \beta) F_1(0) - (\alpha - 2\gamma) F_1^2(0) \\
&\quad - 8F_1(0)F_3(0) \int_0^\infty \left(\alpha - \frac{d}{d\theta} [\log \theta h(t\theta)] \Big|_{\theta=1} \right) [1 - H(t\nu)] h(t) dt.
\end{aligned}$$

Derivation of the expression for I_5 follows similarly.

The expressions $I_1 - I_5$ in (4.2.6) are evaluated in the next Section using exponential, folded standard normal and folded logistic densities for $h(\cdot)$. For any other desired density, the expressions can be calculated similarly.

4.4 Estimation of Optimal Weights

In this section, expressions for $I_1 - I_5$ given in (4.2.6) are derived using the results of Theorems 4.3.1–4.3.3 for three forms of $2h(\cdot)$ – exponential, folded standard normal and folded logistic distribution.

Exponential Distribution

Notice that the exponential distribution is the folded double exponential distribution. By our definition,

$$\begin{aligned} 2[1 - H(t\theta)] &= e^{-t\theta}, & \text{for } t > 0, \\ 2\theta h(t\theta) &= \theta e^{-t\theta}. \end{aligned}$$

Therefore,

$$\frac{d}{d\theta} [\log \theta h(t\theta)] \Big|_{\theta=1} = 1 - t. \quad (4.4.1)$$

Using (4.4.1) we can evaluate β , γ and other necessary constants. We get,

$$\begin{aligned} \beta &= -2 \int_0^\infty \frac{d}{d\theta} [\log \theta h(t\theta)] \Big|_{\theta=1} h(t) dt \\ &= - \int_0^\infty (1 - t) e^{-t} dt \\ &= 0, \end{aligned}$$

so that,

$$\begin{aligned} I_1 &= \int_0^\infty (A_1(t) - A_2(t)) f_1(t) dt \\ &= \alpha F_1(0), \\ I_2 &= \int_0^\infty (A_3(t) - A_4(t)) f_3(t) dt \\ &= \alpha \nu F_3(0). \end{aligned} \quad (4.4.2)$$

Now, γ is given by

$$\begin{aligned}\gamma &= 4 \int \frac{d}{d\theta} [\log \theta h(t\theta)] \Big|_{\theta=1} [1 - H(t)] h(t) dt \\ &= \int (1 - t) e^{-2t} dt \\ &= \frac{1}{4}.\end{aligned}$$

Therefore,

$$\begin{aligned}I_3 &= \int_0^\infty (A_1(t) - A_2(t)) \left(1 - \frac{F_1(t)}{F_1(0)}\right) f_1(t) dt \\ &= \frac{1}{2} \alpha F_1(0) + \frac{1}{4} F_1(0).\end{aligned}\tag{4.4.3}$$

Similarly, to evaluate I_4 and I_5 , we note that

$$\begin{aligned}&8 \int \left(\alpha - \frac{d}{d\theta} [\log \theta h(t\theta)] \Big|_{\theta=1} \right) [1 - H(t\nu)] h(t) dt \\ &= 2 \int (\alpha - 1 + t) e^{-\nu t} e^{-t} dt \\ &= 2 \int (\alpha - 1 + t) e^{-t(1+\nu)} dt \\ &= 2 \frac{\alpha - 1}{1 + \nu} + 2 \frac{1}{(1 + \nu)^2},\end{aligned}$$

$$\begin{aligned}&4 \int \left(\alpha\nu - \frac{d}{d\theta} [\log \nu \theta h(t\nu\theta)] \Big|_{\theta=1} \right) [1 - H(t)] h(t\nu) dt \\ &= \int (\alpha\nu - 1 + \nu t) e^{-t} e^{-\nu t} \nu dt \\ &= \nu \left(\frac{\alpha\nu - 1}{1 + \nu} + \frac{1}{(1 + \nu)^2} \right),\end{aligned}$$

and

$$\begin{aligned}&4 \int \left(\alpha\nu - \frac{d}{d\theta} [\log \theta h(t\nu\theta)] \Big|_{\theta=1} \right) [1 - H(t\nu)] \nu h(t\nu) dt \\ &= \int (\alpha\nu - 1 + \nu t) e^{-\nu t} e^{-\nu t} \nu dt \\ &= \frac{\alpha - 1}{2} + \frac{1}{4}.\end{aligned}$$

Therefore,

$$\begin{aligned}
 I_4 &= \int (A_1(t) - A_2(t)) (1 - F(t)) f_1(t) dt \\
 &= \alpha F_1(0) - \left(\alpha - \frac{1}{2}\right) F_1^2(0) - 2F_1(0)F_3(0) \left[\frac{\alpha - 1}{1 + \nu} + \frac{1}{(1 + \nu)^2} \right], \quad (4.4.4)
 \end{aligned}$$

and

$$\begin{aligned}
 I_5 &= \int (A_3(t) - A_4(t)) \left(1 - \frac{1}{2}F(t)\right) f_3(t) dt \\
 &= \alpha \nu F_3(0) - \left(\frac{\alpha \nu}{2} - \frac{1}{4}\right) F_3^2(0) - \nu F_1(0)F_3(0) \left[\frac{\alpha \nu - 1}{1 + \nu} + \frac{\nu}{(1 + \nu)^2} \right]. \quad (4.4.5)
 \end{aligned}$$

Folded Standard Normal Distribution

For folded standard normal distribution, by our definition,

$$2[1 - H(t\theta)] = \int_t^\infty \frac{2\theta}{\sqrt{2\pi}} e^{-\frac{1}{2}u^2\theta^2} du, \quad \text{for } t > 0,$$

so the corresponding density is

$$2\theta h(t\theta) = \frac{2\theta}{\sqrt{2\pi}} e^{-\frac{1}{2}t^2\theta^2}.$$

Therefore,

$$\frac{d}{d\theta} [\log \theta h(t\theta)] \Big|_{\theta=1} = 1 - t^2. \quad (4.4.6)$$

Using (4.4.6) we can evaluate β , γ and other necessary terms in Theorems 4.3.1–4.3.3.

First we have,

$$\beta = -2 \int \frac{d}{d\theta} [\log \theta h(t\theta)] \Big|_{\theta=1} h(t) dt$$

$$\begin{aligned}
&= -2 \int (1-t^2) \frac{1}{\sqrt{2\pi}} e^{-\frac{1}{2}t^2} \\
&= 0,
\end{aligned}$$

so that,

$$\begin{aligned}
I_1 &= \alpha F_1(0), \\
I_2 &= \alpha \nu F_3(0).
\end{aligned} \tag{4.4.7}$$

Now, γ is given by

$$\begin{aligned}
\gamma &= 4 \int \frac{d}{d\theta} [\log \theta h(t\theta)] \Big|_{\theta=1} [1 - H(t)] h(t) dt \\
&= 4 \int (1-t^2) \left[\int_t^\infty \frac{1}{\sqrt{2\pi}} e^{-\frac{1}{2}u^2} du \right] \frac{1}{\sqrt{2\pi}} e^{-\frac{1}{2}t^2} dt \\
&= 4 \int \left[\int_t^\infty \frac{1}{\sqrt{2\pi}} e^{-\frac{1}{2}u^2} du \right] \frac{1}{\sqrt{2\pi}} e^{-\frac{1}{2}t^2} dt \\
&\quad - 4 \int \left[\int_t^\infty \frac{1}{\sqrt{2\pi}} e^{-\frac{1}{2}u^2} du \right] \frac{t^2}{\sqrt{2\pi}} e^{-\frac{1}{2}t^2} dt \\
&= 0.5 - 0.181616 \\
&= 0.31838 \\
&\simeq \frac{1}{\pi}.
\end{aligned}$$

Therefore,

$$\begin{aligned}
I_3 &= \int_0^\infty (A_1(t) - A_2(t)) \left(1 - \frac{F_1(t)}{F_1(0)} \right) f_1(t) dt \\
&= \frac{1}{2} \alpha F_1(0) + \frac{1}{\pi} F_1(0).
\end{aligned} \tag{4.4.8}$$

Expressions for I_4 and I_5 do not have closed forms for folded standard normal distributions. After estimating the value of ν , the values have to be evaluated numerically.

Folded Logistic Distribution

For folded logistic distribution, by our definition,

$$\begin{aligned} 2[1 - H(t\theta)] &= \frac{2e^{-t\theta}}{1 + e^{-t\theta}}, & \text{for } t > 0, \\ 2\theta h(t\theta) &= \frac{2\theta e^{-t\theta}}{(1 + e^{-t\theta})^2}. \end{aligned}$$

Therefore,

$$\frac{d}{d\theta} [\log \theta h(t\theta)] \Big|_{\theta=1} = 1 - t + \frac{2te^{-t}}{(1 + e^{-t})}. \quad (4.4.9)$$

Using (4.4.9) we can evaluate β , γ and other necessary terms in Theorems 4.3.1–4.3.3.

First, β is given by

$$\begin{aligned} \beta &= -2 \int \frac{d}{d\theta} [\log \theta h(t\theta)] \Big|_{\theta=1} h(t) dt \\ &= -2 \int h(t) dt - 2 \int \left(\frac{2te^{-t}}{(1 + e^{-t})} - t \right) \frac{e^{-t}}{(1 + e^{-t})^2} dt \\ &= -1 + 2 \times 0.443147 \\ &= -0.113706. \end{aligned}$$

Therefore,

$$\begin{aligned} I_1 &= \alpha F_1(0) - 0.113706, \\ I_2 &= \alpha \nu F_3(0) - 0.113706. \end{aligned} \quad (4.4.10)$$

Now, γ is,

$$\begin{aligned} \gamma &= 4 \int \frac{d}{d\theta} [\log \theta h(t\theta)] \Big|_{\theta=1} [1 - H(t)] h(t) dt \\ &= 4 \int \left(1 - t + \frac{2te^{-t}}{1 + e^{-t}} \right) \frac{e^{-2t}}{(1 + e^{-t})^3} dt \end{aligned}$$

$$\begin{aligned}
&= 0.5 + 4 \int \left(\frac{2te^{-t}}{1 + e^{-t}} - t \right) \frac{e^{-2t}}{(1 + e^{-t})^3} dt \\
&= 0.5 - 4 \times 0.01324 \\
&= 0.44704.
\end{aligned}$$

Therefore

$$\begin{aligned}
I_3 &= \frac{1}{2} \alpha F_1(0) + (0.44704 - 0.113706) F_1(0) \\
&\simeq \frac{1}{2} \alpha F_1(0) + \frac{1}{3} F_1(0).
\end{aligned} \tag{4.4.11}$$

For folded logistic distribution expressions I_4 and I_5 do not have closed forms. After estimating the value of ν , the values of I_4 and I_5 can be evaluated numerically.

4.5 Forms of Estimated Optimal Statistics

In this section the forms of the optimal statistics are presented with estimated optimal weights. Consistent estimators are used for estimating optimal weights in the PR and WL-classes of statistics.

The expressions of estimated optimal WL Sign statistic are given here for exponential, folded standard normal and folded logistic distribution.

Optimal WL Sign Statistic

The WL Sign statistic has the form,

$$\begin{aligned}
T &= (1 - \hat{\lambda}) \frac{1}{\sqrt{2F_1(0)}} \int d(N_1(t) - N_2(t)) + \hat{\lambda} \frac{1}{\sqrt{2F_3(0)}} \int d(N_3(t) - N_4(t)) \\
&= (1 - \hat{\lambda}) T_{su}^* + \hat{\lambda} T_{sc}^*,
\end{aligned}$$

where $\hat{\lambda}$ is an estimate of the optimal λ . This estimate can be obtained as follows.

From Theorem 3.4.3, optimal λ is given by

$$\lambda = \frac{c_{su}}{c_{su} + c_{sc}},$$

where c_{su} and c_{sc} are

$$\begin{aligned} c_{su} &= (\text{efficacy of } T_{su})^{-\frac{1}{2}} \\ &= \frac{\sqrt{2F_1(0)}}{\int (A_1(t) - A_2(t)) f_1(t) dt}, \end{aligned}$$

$$\begin{aligned} c_{sc} &= (\text{efficacy of } T_{sc})^{-\frac{1}{2}} \\ &= \frac{\sqrt{2F_3(0)}}{\int (A_3(t) - A_4(t)) f_3(t) dt}. \end{aligned}$$

A consistent estimate of the optimal λ is given by

$$\hat{\lambda} = \frac{\hat{c}_{su}}{\hat{c}_{su} + \hat{c}_{sc}}.$$

Substituting the estimates of I_1 and I_2 from Section 4.4, we have estimates of \hat{c}_{su} and

\hat{c}_{sc} ,

$$\hat{c}_{su} = \frac{\sqrt{2\hat{F}_1(0)}}{(\alpha + \beta)\hat{F}_1(0)} = \frac{\sqrt{2n}}{(\alpha + \beta)\sqrt{N_{u+}}},$$

and

$$\hat{c}_{sc} = \frac{\sqrt{2\hat{F}_3(0)}}{(\alpha\hat{\nu} + \beta)\hat{F}_3(0)} = \frac{\sqrt{2n}}{(\alpha\hat{\nu} + \beta)\sqrt{N_{c+}}}.$$

Therefore, the simplified form of the estimated optimal WL Sign statistic when $\{|Z_i| \mid i \in B_j\}$, $j = 1, 2, 3, 4$ has exponential or folded standard normal distribution

is given by

$$S - OPT = \left[\int d(N_1(t) - N_2(t)) + \hat{\nu} \int d(N_3(t) - N_4(t)) \right].$$

Similarly, the simplified form of the estimated optimal WL Sign statistic when $\{|Z_i| \mid i \in B_j\} \ j = 1, 2, 3, 4$ has folded logistic distribution, can be shown to be given by

$$S - OPT = \left[\int d(N_1(t) - N_2(t)) + \frac{\alpha \hat{\nu} - 0.113706}{\alpha - 0.113706} \int d(N_3(t) - N_4(t)) \right].$$

The estimated optimal PR statistics are now given when $\{|Z_i| \mid i \in B_j\} \ j = 1, 2, 3, 4$ has exponential, folded standard normal and folded logistic distribution.

Optimal PR Statistic

The PR-class of statistics is represented as

$$\begin{aligned} T &= (1 - \hat{\lambda}) \frac{1}{\sqrt{\frac{2}{3}F_1(0)}} \int (1 - F_u(t)) d(N_1(t) - N_2(t)) \\ &\quad + \hat{\lambda} \frac{1}{\sqrt{2F_3(0)}} \int d(N_3(t) - N_4(t)) \\ &= (1 - \hat{\lambda}) T_{1u}^* + \hat{\lambda} T_{1c}^*. \end{aligned}$$

To estimate optimal value of λ we need,

$$\begin{aligned} c_{1u} &= (\text{efficacy of } T_{1u})^{-\frac{1}{2}} \\ &= \frac{\sqrt{\frac{2}{3}F_1(0)}}{\int (A_1(t) - A_2(t)) (1 - F_u(t)) f_1(t) dt}, \end{aligned}$$

and

$$\begin{aligned} c_{1c} &= (\text{efficacy of } T_{1c})^{-\frac{1}{2}} \\ &= \frac{\sqrt{2F_3(0)}}{\int (A_3(t) - A_4(t)) f_3(t) dt}. \end{aligned}$$

Substituting expressions of I_3 and I_4 from Section 4.4, we have estimates of c_{1u} and c_{1c} ,

$$\hat{c}_{1u} = \frac{\sqrt{\frac{2}{3}\hat{F}_1(0)}}{(\frac{1}{2}\alpha + \beta + \gamma) F_1(0)} = \frac{\sqrt{\frac{2}{3}n}}{(\frac{1}{2}\alpha + \beta + \gamma) \sqrt{N_{u+}}}$$

and

$$\hat{c}_c = \frac{\sqrt{2\hat{F}_3(0)}}{(\alpha\hat{\nu} + \beta)\hat{F}_3(0)} = \frac{\sqrt{2n}}{(\alpha\hat{\nu} + \beta)\sqrt{N_{c+}}}.$$

Therefore, the simplified form of the estimated optimal PR statistic when $\{|Z_i| \mid i \in B_j\}$ for $j = 1, 2, 3, 4$ has exponential distribution, is given by

$$\begin{aligned} PR - OPT &= \left[\int (1 - F_u(t)) d(N_1(t) - N_2(t)) \right. \\ &\quad \left. + \frac{\alpha\nu}{3(\frac{\alpha}{2} + \frac{1}{4})} \int d(N_3(t) - N_4(t)) \right]. \end{aligned}$$

The simplified form of the estimated optimal PR statistic when $\{|Z_i| \mid i \in B_j\}$ for $j = 1, 2, 3, 4$ has folded standard normal distribution is given by

$$\begin{aligned} PR - OPT &= \left[\int (1 - F_u(t)) d(N_1(t) - N_2(t)) \right. \\ &\quad \left. + \frac{\alpha\nu}{3(\frac{\alpha}{2} + \frac{1}{\pi})} \int d(N_3(t) - N_4(t)) \right]. \end{aligned}$$

The simplified form of the estimated optimal PR statistic when $\{|Z_i| \mid i \in B_j\}$ for $j = 1, 2, 3, 4$ has folded logistic distribution is given by

$$PR - OPT = \left[\int (1 - F_u(t)) d(N_1(t) - N_2(t)) + \frac{\alpha\nu}{3(\frac{\alpha}{2} + \frac{1}{3})} \int d(N_3(t) - N_4(t)) \right].$$

The estimated optimal WL Wilcoxon signed rank statistic when $\{|Z_i| \mid i \in B_j\}$ for $j = 1, 2, 3, 4$ has exponential distribution is given next.

Optimal WL Wilcoxon Signed Rank Statistic

WL Wilcoxon Signed rank statistic is given by

$$\begin{aligned} T &= (1 - \hat{\lambda}) \frac{1}{\sqrt{2 \int (1 - F(t))^2 f_1(t) dt}} \int (1 - F(t)) d(N_1(t) - N_2(t)) \\ &\quad + \hat{\lambda} \frac{1}{\sqrt{2 \int (1 - \frac{1}{2}F(t))^2 f_3(t) dt}} \int (1 - \frac{1}{2}F(t)) d(N_3(t) - N_4(t)) \\ &= (1 - \hat{\lambda}) T_{wu}^* + \hat{\lambda} T_{wc}^*. \end{aligned}$$

Here c_{wu} and c_{wc} are given by

$$\begin{aligned} c_{wu} &= (\text{efficacy of } T_{wu})^{-\frac{1}{2}} \\ &= \frac{\sqrt{2 \int (1 - F(t))^2 f_1(t) dt}}{\int (A_1(t) - A_2(t)) (1 - F(t)) f_1(t) dt}, \end{aligned}$$

and

$$c_{wc} = (\text{efficacy of } T_{wc})^{-\frac{1}{2}}$$

$$= \frac{\sqrt{\int (1 - \frac{1}{2}F(t))^2 f_3(t)dt}}{\int (A_3(t) - A_4(t))(1 - \frac{1}{2}F(t)) f_3(t)dt}.$$

In Section 4.3, we have mentioned that I_4 and I_5 given in (4.2.6) do not have closed forms when folded logistic and folded standard normal densities are used for $h(\cdot)$.

Using Theorem 4.3.3 and Proposition 2.1 in Dabrowska, (1990), the estimates of c_{wu} and c_{wc} necessary to construct an estimated optimal statistic for the PR-class are given below, with exponential density used for $h(\cdot)$. Recall that $\hat{S}(t)$ is Kaplan-Meier survival function as described in Chapter 2.

$$\begin{aligned}\hat{c}_{wu} &= \frac{n(\sum_{i=1}^2 \int (1 - \hat{S}(t))^2 dN_i)^{0.5}}{\alpha N_{u+} - (\alpha - 0.5)N_{u+}^2 + 2N_{u+}N_{c+}[\frac{1-\alpha}{\hat{\nu}+1} - \frac{1}{(\hat{\nu}+1)^2}]} \\ \hat{c}_{wc} &= \frac{n(\sum_{i=3}^4 \int (1 - 0.5\hat{S}(t))^2 dN_i)^{0.5}}{\hat{\nu}\alpha N_{c+} - (0.5\hat{\nu}\alpha - 0.25)N_{c+}^2 + 2N_{u+}N_{c+}[\frac{1-\hat{\nu}\alpha}{\hat{\nu}+1} - \frac{1}{(\hat{\nu}+1)^2}]}.\end{aligned}$$

The optimal WL Wilcoxon signed rank statistic when $\{|Z_i| \mid i \in B_j\}$ for $j = 1, 2, 3, 4$ has exponential distribution is,

$$WL - OPT = (1 - \hat{\lambda}) T_{wu}^* + \hat{\lambda} T_{wc}^*,$$

where

$$\hat{\lambda} = \frac{\hat{c}_{wu}}{\hat{c}_{wu} + \hat{c}_{wc}}.$$

For any other special case of $h(\cdot)$, optimal statistics of the PR or WL-class can be derived by following similar procedure as shown in Sections 4.3 and 4.4.

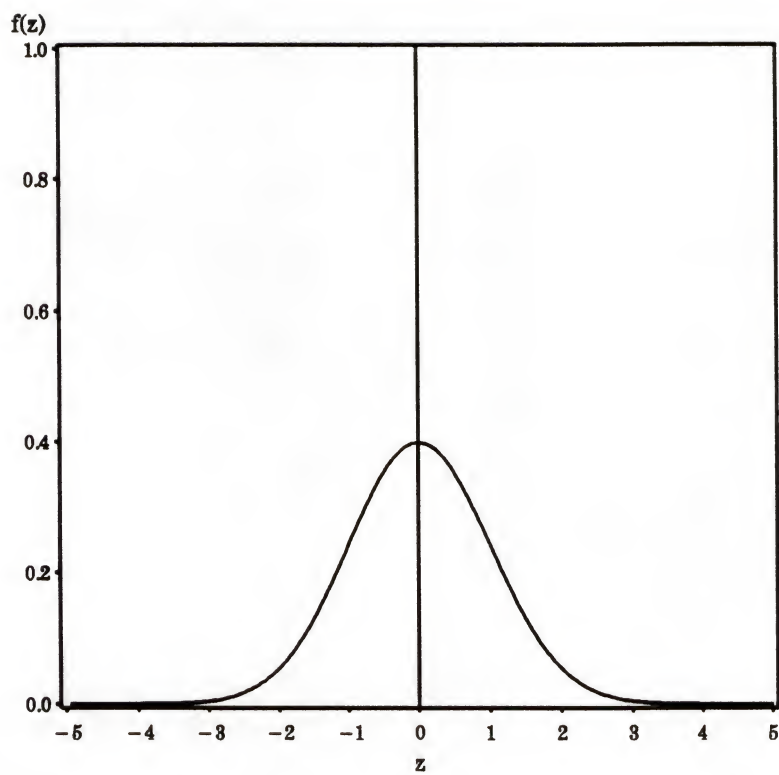


Fig 4.1. Standard Normal Density.

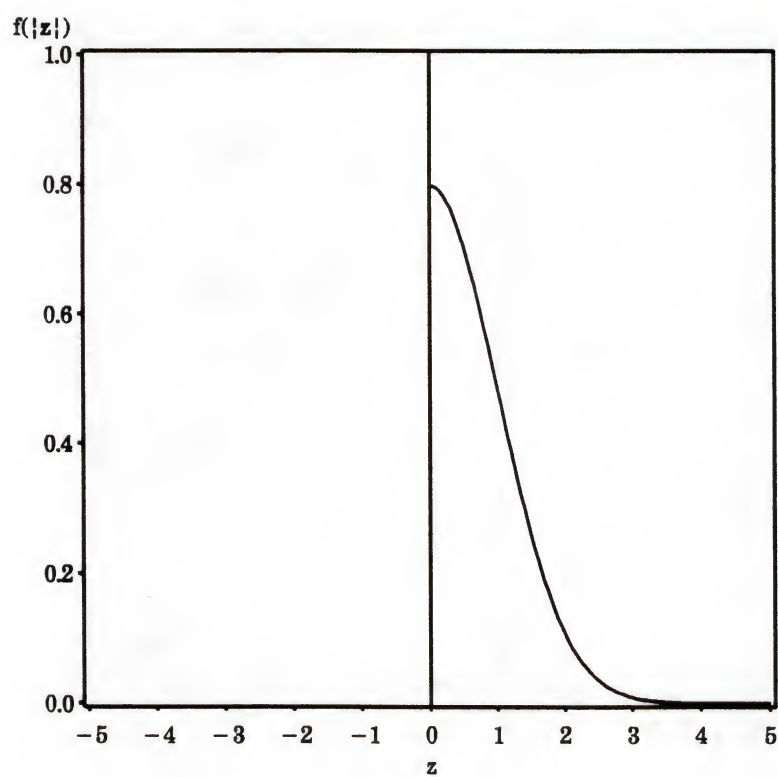


Fig 4.2. Folded Standard Normal Density.

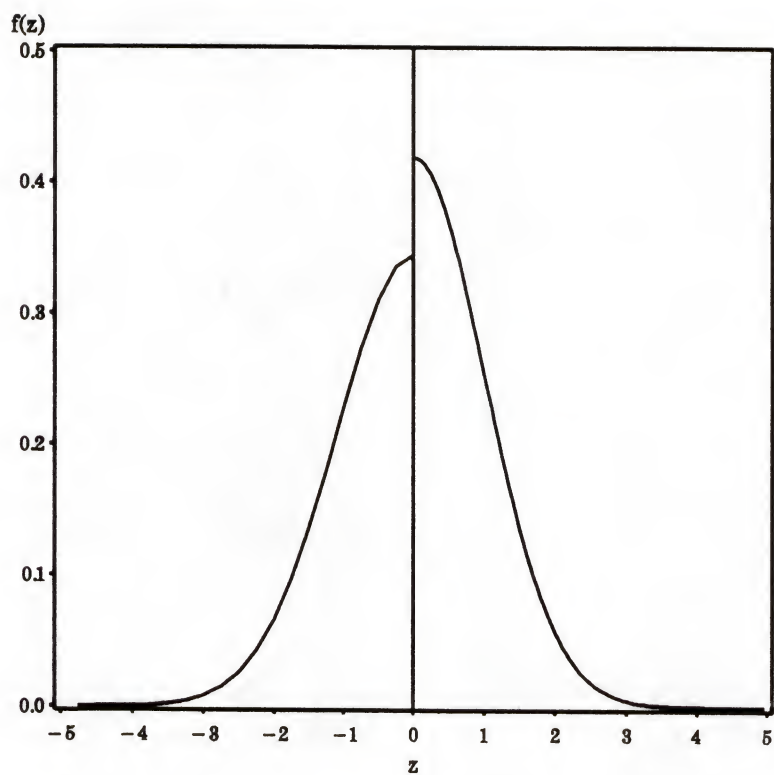


Fig 4.3. $f(z_i | i \in B_1 \cup B_2)$ for $\theta = 1.1$, using
Folded Standard Normal Density for $h(\cdot)$.

CHAPTER 5

SIMULATION RESULTS

5.1 Introduction

The expressions of the estimated optimal weights in the PR and WL-classes are given in Chapter 4 for exponential, folded standard normal and folded logistic distributions. Some of the integrals involved in determining optimal weights for the WL Wilcoxon signed rank statistic do not have closed forms. Therefore, the estimated optimal weights in these cases could not be listed for all the densities we considered. In this chapter, we compare powers of several relevant statistics using a simulation study. The results of simulation are presented in Section 5.2.

5.2 Simulation Study

A simulation study was performed using the model given in (4.2.1) and (4.2.11). Three functional forms, each with support $[0, \infty)$, were considered for $2h(\cdot)$ defined at (4.2.1). They were

the exponential,

$$2\nu h(t|\nu) = \nu e^{-\nu t},$$

the folded standard normal distribution,

$$2\nu h(t|\nu) = 2\frac{\nu}{\sqrt{2\pi}}e^{-\frac{1}{2}\nu^2 t^2},$$

and the folded logistic,

$$2\nu h(t|\nu) = 2 \frac{\nu e^{-\nu t}}{(1 + e^{-\nu t})^2}.$$

Two values $\nu = 0.75$ and $\nu = 1.25$ were considered for the censoring parameter ν . Our choice for the values for ν was based on the following considerations.

1. When $\nu < 1.0$, under H_0 , the standard deviation of the distribution of censored Z is smaller than that of an uncensored Z . This implies that, in most singly censored pairs, the censored observations are not very high compared to the true survival times of the uncensored observations. Thus, $\nu < 1$ implies that the censored observations are not very high compared to the uncensored observations in singly censored pairs. We choose $\nu = 0.75$ to study this case.
2. When $\nu > 1.0$, the standard deviation of the distribution of censored Z is greater than that of an uncensored Z . So, in many of the singly censored pairs, censored observations are much larger than the corresponding uncensored survival times. Thus, $\nu > 1$ implies that the censored observations are much higher than the uncensored observations in singly censored pairs. We chose $\nu = 1.25$ to study this case.

For the odds ratio in (4.2.11), we selected $g(\theta) = \alpha(\theta - 1)$, and $\alpha = 0.6$. Therefore,

$$\frac{F_{1\theta}(0)}{F_{2\theta}(0)} = \exp(\alpha(\theta - 1)),$$

and

$$\frac{F_{3\theta}(0)}{F_{4\theta}(0)} = \exp(\alpha\nu(\theta - 1)).$$

Power and size of a 0.05 level test based on each statistic was estimated by generating 500 random samples of sizes $n = 100$ and $n = 40$. The alternatives were

specified by selected values of θ . Recall that for certain statistics (e.g. PR-OPT) estimation of optimal weights require specification of a value for α . During simulation, by specifying a value for α alternative conditional distributions of the $|Z_i|$ are also specified. Therefore, we are interested in studying the effect of using a wrong value of α for estimation of optimal weights. Powers of the test statistics were estimated when the optimal weights were calculated using a value of α different from 0.6. Two of the values specified were higher than the $\alpha = 0.6$ used for generating samples. The values were $\alpha = 1$ and $\alpha = 1.5$. The other two specified values were smaller than 0.6, $\alpha = 0.5$ and $\alpha = 0.3$. These values for rate α were chosen arbitrarily.

Samples were generated such that $P(i \in B_1 \cup B_2) = 0.3$, $P(i \in B_3 \cup B_4) = 0.6$ and $P(i \in B_5) = 0.1$. Thus the expected proportion of uncensored, singly censored and doubly censored pairs were, respectively, 30%, 60% and 10%.

Actual simulation of random samples was performed in two stages. In the first stage a random sample of size n was selected from an uniform $[0, 1]$ population. Let the observations be denoted by u_i , $i = 1, 2, \dots, 100$. In the second stage, the sample of the differences (z) were generated. The observations from the uniform distribution are used to determine the censoring status and the sign of the differences. Here is an example of how it is done under the case of null hypothesis ($\theta = 1$) where the expected number of positive differences is same as the number of negative differences among both censored and uncensored differences. Since the probability is 0.60 that an observation is from an uncensored pair, we let

$$f(|z_i| \mid i \in B_1) = h(|z_i|), \quad \text{if } 0 < u_i < 0.30,$$

and

$$f(|z_i| \mid i \in B_1) = h(|z_i| \theta), \quad \text{if } 0.30 < u_i < 0.60.$$

Similarly to generate censored differences,

$$f(|z_i| \mid i \in B_3) = h(|z_i| \nu), \quad \text{if } 0.60 < u_i < 0.75,$$

and

$$f(|z_i| \mid i \in B_4) = h(|z_i| \nu \theta), \quad \text{if } 0.75 < u_i < 0.90.$$

From each sample, the following statistics were calculated.

1. PR-EQ : PR statistic with equal weights.
2. PR-STD : PR statistic with weights proportional to standard deviations.
3. PR-OPT : PR statistic with optimal weights.
4. PR-OPT(1), PR-OPT(2), PR-OPT(3) and PR-OPT(4) : PR statistic with optimal weights and $\alpha = 1.0$, $\alpha = 1.5$, $\alpha = 0.30$ and $\alpha = 0.10$ respectively.
5. S-EQ : WL Sign statistic with equal weights.
6. S-STD : WL Sign statistic with weights proportional to standard deviations.
7. S-OPT : WL Sign statistic with optimal weights.
8. S-OPT(1), S-OPT(2), S-OPT(3) and S-OPT(4) : WL Sign statistic with optimal weights and $\alpha = 1.0$, $\alpha = 1.5$, $\alpha = 0.30$ and $\alpha = 0.10$ respectively.
9. W-EQ : WL Wilcoxon signed rank statistic with equal weights.
10. W-STD : WL Wilcoxon signed rank statistic with weights proportional to standard deviations.

Notice that in this list of statistics considered for simulation study, optimal WL Wilcoxon signed rank statistic (W-OPT) was not included. This statistic was left out on purpose because of two reasons. In Chapter 3, we have seen that when there is association (which is usually the case with bivariate data) within the pairs, the efficacy of PR-OPT is always higher than the efficacy of W-OPT. Secondly, the form of the estimated W-OPT is very complicated and does not have a closed form in most cases we are considering. Consequently, computation of WL-OPT involves a complicated procedure which requires enormous computer time. Considering all these factors we decided not to include W-OPT in the simulation study.

For estimating power of the optimal statistics, the following test procedure was used. If any of the estimated odds ratios are equal to 1, then the statistic with weights proportional to standard deviations is used instead of the optimal statistic. This procedure was used because when any of the estimated odds ratios are 1, estimation of optimal weights is not possible.

First let us look at the powers of tests when sample size is large ($n = 100$). These cases are presented in the Tables 5.1–5.6. Notice that in each table, estimated level of test is within $0.05 - 2\sqrt{\frac{(0.05)(0.95)}{500}}$ and $0.05 + 2\sqrt{\frac{(0.05)(0.95)}{500}}$ i.e. within (0.0305, 0.0695).

In Table 5.1, $(|Z_i| \mid i \in B_j)$, $j = 1, 2, 3, 4$, have exponential distributions, and $\nu = 0.75$. Among the PR-class of statistics, PR-EQ performed better than PR-STD. But, there was almost 30% (power of PR-EQ=0.302 vs power of PR-OPT=0.410) improvement of power near the null hypothesis when PR-OPT was used instead of PR-EQ. As we move further away from the null hypothesis, the improvement in power resulting from the use of the optimal statistic in any class is not so much. This is as expected because any reasonable statistic should have high power when the alternative hypothesis is very far away from the null hypothesis. Among Sign statistics, S-STD performed better than S-EQ. Near the null hypothesis, there was

40% improvement of power by using S-OPT. Although powers of W-EQ and W-STD were higher than that of S-OPT, in some cases, they were always less than that of PR-OPT.

As we have mentioned earlier, we are interested to see how powers of optimal statistics are affected by wrong specification of α . From each table, it is clear that, for S-OPT powers are not at all affected by specifying values of α that are different from 0.6. Recall that $\alpha = 0.6$ was used in specifying the alternative hypothesis. For PR-OPT, when the value of α specified for calculation of optimal weights is lower than the true α , in general there is some loss of power.

In Table 5.2 ($|Z_i| \mid i \in B_j$), $j = 1, 2, 3, 4$, have exponential distributions and $\nu = 1.25$. The powers of W-EQ and W-STD were sometimes higher than that for PR-OPT and S-OPT. Although near the null hypothesis there is almost 18% (power of PR-EQ=0.372 vs power of PR-OPT=0.438) increase in power resulting from the use of the PR-OPT than any other statistics in the PR-class, the powers of W-EQ and W-STD are not very low compared to PR-OPT at alternatives substantially different from the H_0 . In this case, either PR-OPT, W-EQ or W-STD should be used.

In Table 5.3, the case of ($|Z_i| \mid i \in B_j$), $j = 1, 2, 3, 4$, with folded standard normal distributions and $\nu = 0.75$ is presented. Here, members of the PR-class are performing very well. Powers of W-EQ and W-STD both were low compared to the powers of the rest of the statistics in our list. In this case, the power of PR-STD is higher than that of PR-EQ and near H_0 , the improvement in power resulting from the use of PR-OPT is almost 30%. Here, the choice of statistic to be used is PR-OPT.

In Table 5.4, where ($|Z_i| \mid i \in B_j$), $j = 1, 2, 3, 4$, have folded standard normal distributions and $\nu = 1.25$. The performances of W-EQ and W-STD were poor compared to the performance of other statistics in the list. It appears from Tables 5.3–5.4, that when the conditional distribution of absolute differences $|Z_i|$ have folded

standard normal distributions, the powers of both W-EQ and W-STD will be low and the choice of statistic to be used is PR-OPT.

In Table 5.5, when $(|Z_i| \mid i \in B_j)$, $j = 1, 2, 3, 4$, have folded logistic distributions and $\nu = 0.75$, members of the PR-class have powers higher than the statistics in WL-class. In this situation the recommended statistic is PR-OPT. There is almost 40% increase in power near the null hypothesis resulting from the use of the optimal statistic in the PR-class instead of any other member from the same class. Although the improvement in power resulting from using S-OPT instead of S-EQ or S-STD is more than 40%, power of S-OPT is still much lower than the power of PR-OPT.

In Table 5.5, when $(|Z_i| \mid i \in B_j)$, $j = 1, 2, 3, 4$, have folded logistic distributions and $\nu = 1.25$, the power of PR-OPT is higher than the power of the other statistics. But, in this case, W-EQ and W-STD have higher powers than S-EQ and S-STD.

Now we consider the case of moderate ($n = 40$) sample sizes. Here also, in each table, estimated level of test is within $0.05 - 2\sqrt{\frac{(0.05)(0.95)}{500}}$ and $0.05 + 2\sqrt{\frac{(0.05)(0.95)}{500}}$ i.e. within (0.0305, 0.0695).

For $(|Z_i| \mid i \in B_j)$, $j = 1, 2, 3, 4$, having exponential distributions, when $\nu = 0.75$, PR-OPT is performing well overall. Also, both W-EQ and W-STD are performing better than the Sign statistics, even better than the optimal Sign test as we move away from the null hypothesis. From Table 5.8, when $\nu = 1.25$, PR-OPT has high power near H_0 , but W-EQ and W-STD also are performing very well. Also, it is clear that the power of PR-OPT is not very high compared to W-EQ when $\theta > 1.33$. In this case the choice is between PR-OPT and W-EQ.

Like the case of $n = 100$, when $(|Z_i| \mid i \in B_j)$, $j = 1, 2, 3, 4$, have folded standard normal distributions, W-EQ and W-STD are performing poorly compared to the

other statistics. From Tables 5.9 and 5.10, the statistic which performs best is PR-OPT. The same is true for both the values of ν when conditional distribution of $|Z_i|$ has folded logistic density.

Therefore, it appears that in most of the cases we have considered, PR-OPT emerges as the best statistic. Also, recall that for any member of the PR-class, an exact test can be performed for small samples. Only, when $(|Z_i| | i \in B_j), j = 1, 2, 3, 4$, have exponential distribution, powers of W-EQ and W-STD are very close to that of PR-OPT.

Table 5.1. Power of the tests for Exponential distribution with $\alpha = 0.6$,
 $\nu = 0.75$. and sample size = 100.

Tests	$\theta = 1.00$	$\theta = 1.33$	$\theta = 1.66$	$\theta = 2.00$
PR-EQ	0.040	0.302	0.656	0.900
PR-STD	0.040	0.286	0.628	0.888
PR-OPT	0.040	0.410	0.740	0.964
PR-OPT(1)	0.040	0.410	0.742	0.964
PR-OPT(2)	0.040	0.414	0.736	0.964
PR-OPT(3)	0.040	0.408	0.740	0.964
PR-OPT(4)	0.040	0.374	0.732	0.960
S-EQ	0.042	0.244	0.516	0.800
S-STD	0.052	0.260	0.538	0.828
S-OPT	0.052	0.380	0.634	0.898
S-OPT(1)	0.052	0.380	0.634	0.898
S-OPT(2)	0.052	0.380	0.634	0.898
S-OPT(3)	0.052	0.380	0.634	0.898
S-OPT(4)	0.052	0.380	0.634	0.898
W-EQ	0.056	0.322	0.734	0.942
W-STD	0.044	0.316	0.702	0.924

Table 5.2. Power of the tests for Exponential distribution with $\alpha = 0.6$,
 $\nu = 1.25$. and sample size = 100.

Tests	$\theta = 1.00$	$\theta = 1.33$	$\theta = 1.66$	$\theta = 2.00$
PR-EQ	0.040	0.372	0.794	0.972
PR-STD	0.040	0.362	0.782	0.962
PR-OPT	0.040	0.438	0.830	0.976
PR-OPT(1)	0.040	0.440	0.828	0.976
PR-OPT(2)	0.040	0.446	0.826	0.976
PR-OPT(3)	0.040	0.436	0.826	0.976
PR-OPT(4)	0.040	0.388	0.784	0.974
S-EQ	0.042	0.294	0.650	0.914
S-STD	0.052	0.300	0.642	0.920
S-OPT	0.052	0.406	0.726	0.942
S-OPT(1)	0.052	0.406	0.726	0.942
S-OPT(2)	0.052	0.406	0.726	0.942
S-OPT(3)	0.052	0.406	0.726	0.942
S-OPT(4)	0.052	0.406	0.726	0.942
W-EQ	0.056	0.410	0.844	0.982
W-STD	0.042	0.402	0.848	0.982

Table 5.3. Power of the tests for Folded Standard Normal distribution with $\alpha = 0.6$, $\nu = 0.75$. and sample size = 100.

Tests	$\theta = 1.00$	$\theta = 1.33$	$\theta = 1.66$	$\theta = 2.00$
PR-EQ	0.038	0.356	0.732	0.942
PR-STD	0.038	0.316	0.694	0.920
PR-OPT	0.038	0.458	0.832	0.978
PR-OPT(1)	0.038	0.460	0.818	0.978
PR-OPT(2)	0.038	0.464	0.814	0.978
PR-OPT(3)	0.038	0.452	0.834	0.978
PR-OPT(4)	0.038	0.418	0.828	0.978
S-EQ	0.042	0.244	0.516	0.800
S-STD	0.052	0.260	0.538	0.828
S-OPT	0.052	0.380	0.634	0.898
S-OPT(1)	0.052	0.380	0.634	0.898
S-OPT(2)	0.052	0.380	0.634	0.898
S-OPT(3)	0.052	0.380	0.634	0.898
S-OPT(4)	0.052	0.380	0.634	0.898
W-EQ	0.044	0.202	0.492	0.754
W-STD	0.050	0.202	0.474	0.740

Table 5.4. Power of the tests for Folded Standard Normal distribution with $\alpha = 0.6$, $\nu = 1.25$, and sample size = 100.

Tests	$\theta = 1.00$	$\theta = 1.33$	$\theta = 1.66$	$\theta = 2.00$
PR-EQ	0.038	0.420	0.838	0.990
PR-STD	0.038	0.404	0.826	0.982
PR-OPT	0.038	0.480	0.874	0.992
PR-OPT(1)	0.038	0.478	0.868	0.992
PR-OPT(2)	0.038	0.480	0.862	0.992
PR-OPT(3)	0.038	0.484	0.876	0.992
PR-OPT(4)	0.038	0.436	0.858	0.992
S-EQ	0.042	0.294	0.650	0.914
S-STD	0.052	0.300	0.642	0.920
S-OPT	0.052	0.406	0.726	0.942
S-OPT(1)	0.052	0.406	0.726	0.942
S-OPT(2)	0.052	0.406	0.726	0.942
S-OPT(3)	0.052	0.406	0.726	0.942
S-OPT(4)	0.052	0.406	0.726	0.942
W-EQ	0.054	0.274	0.622	0.884
W-STD	0.058	0.266	0.626	0.888

Table 5.5. Power of the tests for Folded Logistic distribution with $\alpha = 0.6$, $\nu = 0.75$. and sample size = 100.

Tests	$\theta = 1.00$	$\theta = 1.33$	$\theta = 1.66$	$\theta = 2.00$
PR-EQ	0.062	0.360	0.680	0.950
PR-STD	0.062	0.334	0.666	0.922
PR-OPT	0.062	0.478	0.798	0.976
PR-OPT(1)	0.062	0.474	0.794	0.976
PR-OPT(2)	0.062	0.468	0.790	0.974
PR-OPT(3)	0.062	0.452	0.794	0.972
PR-OPT(4)	0.062	0.336	0.708	0.940
S-EQ	0.042	0.244	0.516	0.798
S-STD	0.052	0.260	0.538	0.828
S-OPT	0.052	0.380	0.632	0.898
S-OPT(1)	0.052	0.380	0.632	0.898
S-OPT(2)	0.052	0.380	0.632	0.898
S-OPT(3)	0.052	0.380	0.632	0.898
S-OPT(4)	0.052	0.380	0.632	0.898
W-EQ	0.056	0.210	0.478	0.750
W-STD	0.054	0.202	0.476	0.746

Table 5.6. Power of the tests for Folded Logistic distribution with $\alpha = 0.6$, $\nu = 1.25$. and sample size = 100.

Tests	$\theta = 1.00$	$\theta = 1.33$	$\theta = 1.66$	$\theta = 2.00$
PR-EQ	0.062	0.366	0.706	0.956
PR-STD	0.062	0.344	0.690	0.932
PR-OPT	0.062	0.480	0.814	0.976
PR-OPT(1)	0.062	0.474	0.810	0.976
PR-OPT(2)	0.062	0.470	0.806	0.974
PR-OPT(3)	0.062	0.452	0.814	0.974
PR-OPT(4)	0.062	0.334	0.712	0.934
S-EQ	0.042	0.256	0.546	0.818
S-STD	0.052	0.264	0.558	0.844
S-OPT	0.052	0.384	0.662	0.904
S-OPT(1)	0.052	0.384	0.662	0.904
S-OPT(2)	0.052	0.384	0.662	0.904
S-OPT(3)	0.052	0.384	0.662	0.904
S-OPT(4)	0.052	0.384	0.662	0.904
W-EQ	0.050	0.268	0.624	0.874
W-STD	0.052	0.272	0.632	0.876

Table 5.7. Power of the tests for Exponential distribution with $\alpha = 0.6$, $\nu = 0.75$. and sample size = 40.

Tests	$\theta = 1.00$	$\theta = 1.33$	$\theta = 1.66$	$\theta = 2.00$	$\theta = 2.33$
PR-EQ	0.040	0.152	0.364	0.614	0.784
PR-STD	0.042	0.136	0.354	0.580	0.762
PR-OPT	0.042	0.250	0.512	0.706	0.864
PR-OPT(1)	0.042	0.252	0.512	0.708	0.858
PR-OPT(2)	0.042	0.252	0.512	0.712	0.860
PR-OPT(3)	0.042	0.240	0.502	0.704	0.848
PR-OPT(4)	0.042	0.196	0.460	0.680	0.834
S-EQ	0.050	0.138	0.302	0.508	0.676
S-STD	0.050	0.138	0.308	0.540	0.714
S-OPT	0.050	0.214	0.448	0.636	0.776
S-OPT(1)	0.050	0.214	0.448	0.636	0.776
S-OPT(2)	0.050	0.214	0.448	0.636	0.776
S-OPT(3)	0.050	0.214	0.448	0.636	0.776
S-OPT(4)	0.050	0.214	0.448	0.636	0.776
W-EQ	0.040	0.170	0.392	0.638	0.818
W-STD	0.040	0.156	0.378	0.614	0.788

Table 5.8. Power of the tests for Exponential distribution with $\alpha = 0.6$, $\nu = 1.25$. and sample size = 40.

Tests	$\theta = 1.00$	$\theta = 1.33$	$\theta = 1.66$	$\theta = 2.00$	$\theta = 2.33$
PR-EQ	0.040	0.188	0.462	0.730	0.896
PR-STD	0.042	0.182	0.446	0.712	0.898
PR-OPT	0.042	0.266	0.556	0.762	0.892
PR-OPT(1)	0.042	0.268	0.556	0.764	0.894
PR-OPT(2)	0.042	0.270	0.554	0.766	0.894
PR-OPT(3)	0.042	0.252	0.550	0.760	0.890
PR-OPT(4)	0.042	0.202	0.506	0.724	0.870
S-EQ	0.050	0.162	0.380	0.628	0.828
S-STD	0.050	0.152	0.378	0.640	0.828
S-OPT	0.050	0.228	0.478	0.684	0.842
S-OPT(1)	0.050	0.228	0.478	0.684	0.842
S-OPT(2)	0.050	0.228	0.478	0.684	0.842
S-OPT(3)	0.050	0.228	0.478	0.684	0.842
S-OPT(4)	0.050	0.228	0.478	0.684	0.842
W-EQ	0.042	0.214	0.480	0.752	0.918
W-STD	0.040	0.202	0.472	0.746	0.918

Table 5.9. Power of the tests for Folded Standard Normal distribution with $\alpha = 0.6$, $\nu = 0.75$. and sample size = 40.

Tests	$\theta = 1.00$	$\theta = 1.33$	$\theta = 1.66$	$\theta = 2.00$	$\theta = 2.33$
PR-EQ	0.042	0.158	0.394	0.676	0.838
PR-STD	0.040	0.150	0.384	0.626	0.788
PR-OPT	0.040	0.244	0.540	0.784	0.912
PR-OPT(1)	0.040	0.250	0.542	0.784	0.912
PR-OPT(2)	0.040	0.246	0.542	0.780	0.910
PR-OPT(3)	0.040	0.234	0.524	0.782	0.910
PR-OPT(4)	0.040	0.214	0.492	0.760	0.890
S-EQ	0.050	0.138	0.302	0.508	0.676
S-STD	0.050	0.138	0.308	0.540	0.714
S-OPT	0.050	0.214	0.448	0.636	0.776
S-OPT(1)	0.050	0.214	0.448	0.636	0.776
S-OPT(2)	0.050	0.214	0.448	0.636	0.776
S-OPT(3)	0.050	0.214	0.448	0.636	0.776
S-OPT(4)	0.050	0.214	0.448	0.636	0.776
W-EQ	0.056	0.136	0.254	0.456	0.628
W-STD	0.050	0.124	0.246	0.444	0.616

Table 5.10. Power of the tests for Folded Standard Normal distribution with $\alpha = 0.6$, $\nu = 1.25$. and sample size = 40.

Tests	$\theta = 1.00$	$\theta = 1.33$	$\theta = 1.66$	$\theta = 2.00$	$\theta = 2.33$
PR-EQ	0.042	0.206	0.516	0.770	0.936
PR-STD	0.040	0.206	0.490	0.750	0.928
PR-OPT	0.040	0.270	0.588	0.816	0.916
PR-OPT(1)	0.040	0.278	0.592	0.814	0.916
PR-OPT(2)	0.040	0.274	0.594	0.812	0.914
PR-OPT(3)	0.040	0.258	0.578	0.810	0.914
PR-OPT(4)	0.040	0.222	0.542	0.786	0.900
S-EQ	0.050	0.162	0.380	0.628	0.828
S-STD	0.050	0.152	0.378	0.640	0.828
S-OPT	0.050	0.228	0.478	0.684	0.842
S-OPT(1)	0.050	0.228	0.478	0.684	0.842
S-OPT(2)	0.050	0.228	0.478	0.684	0.842
S-OPT(3)	0.050	0.228	0.478	0.684	0.842
S-OPT(4)	0.050	0.228	0.478	0.684	0.842
W-EQ	0.052	0.152	0.338	0.588	0.772
W-STD	0.046	0.150	0.344	0.582	0.786

Table 5.11. Power of the tests for Folded Logistic distribution with $\alpha = 0.6$, $\nu = 0.75$. and sample size = 40.

Tests	$\theta = 1.00$	$\theta = 1.33$	$\theta = 1.66$	$\theta = 2.00$	$\theta = 2.33$
PR-EQ	0.060	0.178	0.400	0.650	0.808
PR-STD	0.060	0.168	0.374	0.620	0.780
PR-OPT	0.060	0.272	0.538	0.762	0.888
PR-OPT(1)	0.060	0.272	0.540	0.764	0.890
PR-OPT(2)	0.060	0.272	0.540	0.760	0.886
PR-OPT(3)	0.060	0.244	0.524	0.752	0.886
PR-OPT(4)	0.060	0.166	0.412	0.642	0.802
S-EQ	0.050	0.138	0.302	0.508	0.676
S-STD	0.050	0.138	0.308	0.540	0.714
S-OPT	0.050	0.214	0.448	0.636	0.776
S-OPT(1)	0.050	0.214	0.448	0.636	0.776
S-OPT(2)	0.050	0.214	0.448	0.636	0.776
S-OPT(3)	0.050	0.214	0.448	0.636	0.776
S-OPT(4)	0.050	0.214	0.448	0.636	0.776
W-EQ	0.052	0.144	0.268	0.458	0.634
W-STD	0.060	0.140	0.250	0.438	0.618

Table 5.12. Power of the tests for Folded Logistic distribution with $\alpha = 0.6$, $\nu = 1.25$. and sample size = 40.

Tests	$\theta = 1.00$	$\theta = 1.33$	$\theta = 1.66$	$\theta = 2.00$	$\theta = 2.33$
PR-EQ	0.060	0.216	0.486	0.774	0.922
PR-STD	0.060	0.218	0.464	0.756	0.906
PR-OPT	0.060	0.292	0.576	0.804	0.898
PR-OPT(1)	0.060	0.298	0.580	0.810	0.902
PR-OPT(2)	0.060	0.300	0.582	0.804	0.902
PR-OPT(3)	0.060	0.262	0.562	0.796	0.892
PR-OPT(4)	0.060	0.174	0.426	0.652	0.774
S-EQ	0.050	0.162	0.380	0.628	0.828
S-STD	0.050	0.152	0.378	0.640	0.828
S-OPT	0.050	0.228	0.478	0.684	0.842
S-OPT(1)	0.050	0.228	0.478	0.684	0.842
S-OPT(2)	0.050	0.228	0.478	0.684	0.842
S-OPT(3)	0.050	0.228	0.478	0.684	0.842
S-OPT(4)	0.050	0.228	0.478	0.684	0.842
W-EQ	0.052	0.170	0.340	0.600	0.786
W-STD	0.062	0.166	0.334	0.574	0.790

5.3 A Worked Example

We consider the leukemia remission data of Freirch et al. (1963) to analyze the difference between the remission times of patients in control and treatment groups. Leukemia patients sharing similar characteristics were paired together and each member of the pair was assigned at random to either treatment or control group. The following table gives remission times in weeks.

Table 5.1. Paired remission times for Leukemia patients. Censored observations are indicated by asterisks.

Obs.	1	2	3	4	5	6	7	8	9	10	11	12	13
Control	1	22	3	2	8	17	2	11	8	12	2	5	4
Treatment	10	7	22*	23	22	6	16	34*	32*	25*	11*	20*	19*

Obs.	14	15	16	17	18	19	20	21
Control	15	8	23	5	11	4	1	8
Treatment	6	17*	35*	6	13	9*	6*	10*

As can be seen from the data, $N_{u+} = 3$, $N_{u-} = 6$, $N_{c+} = 12$, and $N_{c-} = 0$. A consistent estimator of ν is given by

$$\hat{\nu} = \frac{\log N_{c+} - \log N_{c-}}{\log N_{s+} - \log N_{s-}}.$$

Since $N_{c-} = 0$, $\log N_{c-}$ is undefined. In order to estimate ν , we assume that $\log N_{c-} = 0$ and we get $\hat{\nu} = 3.585$.

The normal q-q plots for $\{|z_i| \mid B_j\}$, $j = 1, 2, 3$, indicate that normal distributions fit the differences well within each class. Of course, the small number of observations in each class makes the reliability of q-q analysis somewhat questionable.

After some elementary calculations, we have

$$S - EQ = 3.156, \quad S - STD = 3.273, \quad S - OPT = 3.602.$$

The corresponding one sided p-values are 0.0007, 0.0005 and 0.00015 respectively.

From the PR-class,

$$PR - EQ = 2.952 \quad \text{and} \quad PR - STD = 3.384,$$

and the corresponding one sided p-values are 0.0015 and 0.00035.

Also, we have

$$W - EQ = 2.897 \quad \text{and} \quad W - STD = 3.459$$

with one sided p-values 0.00188 and 0.00027 respectively. All these statistics indicate that the null hypothesis should be rejected.

Now, to calculate the PR-OPT, we have to specify the slope α . Specifying the values of slope as 0.6, 0.8, 1.0, and 1.5, the estimated optimal PR statistics are given by 3.439, 3.476, 3.495 and 3.515. The one sided p-values corresponding to these statistics are 0.00023, 0.00029, 0.00025, and 0.00022 respectively.

Therefore, by using any of the test procedures we reject the null hypothesis of symmetry and conclude the remission times of patients in treatment group is longer than the remission times of patients in control group.

5.4 Conclusions

The simulation study of Section 5.2 compared powers of several statistics for large ($n = 100$) and moderate ($n = 40$) sample sizes. In most of the cases, we considered, optimal statistic in the PR-class emerged as the best statistic in terms of power under the alternatives. Only, when $\{|z_i| \mid B_j\}$, $j = 1, 2, 3$, have exponential distributions, W-EQ or W-STD could be used instead of PR-OPT.

Regarding the optimal statistics, we have seen that the power of PR-OPT is slightly low when the value of α specified in the calculation of optimal weight is lower than the true value of α which characterizes the distribution. Therefore, in selecting a value for α , it is recommended that one chooses a “high” value for α .

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BIOGRAPHICAL SKETCH

Aparna RayChaudhuri was born on February 29, 1964, in Calcutta, India. After finishing high school in 1982 she went to Presidency College, Calcutta. In 1986 she received Bachelor of Science from University of Calcutta with honors in statistics and received P.C. Mahalonobis Award for graduating at the top of the class. She came to graduate school at the University of Florida in Fall 1986 and received the Master of Statistics in 1988. She expects to receive the degree Doctor of Philosophy at the end of summer 1992. She is a member of the Institute of Mathematical Statistics.

While in graduate school Aparna worked as a Teaching Assistant for three years. For the last three years, she has been working as a consultant in the consulting center of the Department of Statistics within the Institute of Food and Agriculture Sciences. Upon graduation, she will be an assistant professor in the Division of Mathematics, Statistics and Computer Science of the University of Texas at San Antonio.

I certify that I have read this study and that in my opinion it conforms to acceptable standards of scholarly presentation and is fully adequate, in scope and quality, as a dissertation for the degree of Doctor of Philosophy.



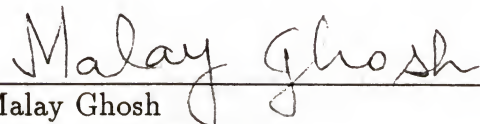
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This dissertation was submitted to the Graduate Faculty of the Department of Statistics in the College of Liberal Arts and Sciences and to the Graduate School and was accepted as partial fulfillment of the requirements for the degree of Doctor of Philosophy.

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